Virtual Investor Day
Welcome

Chris Stevo, Head of Investor Relations
# Agenda

## Introduction: Vision and Strategy
- **Ludwig Hantson**, Ph.D., CEO
- **Aradhana Sarin**, M.D., CFO

## Board of Directors Perspective
- **David Brennan**, Chairman of the Board

## R&D Portfolio Overview
- **John Orloff**, M.D., Head of R&D

## Lead & Expand: Sustainability in Our C5 Business
- **Brian Goff**, Chief Commercial & Global Operations Officer
- **John Orloff**, M.D., Head of R&D

## Q&A Session I

### Highlighted Diversification Opportunities and Novel Platforms in our Portfolio

<table>
<thead>
<tr>
<th>Platform</th>
<th>Presenter(s)</th>
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<tbody>
<tr>
<td>ALXN1840 in Wilson Disease</td>
<td><strong>John Orloff</strong>, M.D., Head of R&amp;D &amp; <strong>Brian Goff</strong>, Chief Commercial &amp; Global Operations Officer</td>
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<td>CAEL-101 in AL Amyloidosis</td>
<td><strong>Cristina Quarta</strong>, M.D., Ph.D., CAEL-101 Clinical Development Lead</td>
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</table>
| Factor D Platform (ALXN2040 & ALXN2050) | **Gianluca Pirozzi**, M.D., Ph.D., Head of Clinical Development and Translational Sciences  
**Anita Hill**, M.D., Ph.D., Hematology Global Medical Affairs Lead  
**Darius Moshfeghi**, M.D., Clinical Consultant GA Program & Professor, Stanford University |
| FcRn Platform (ALXN1830) | **Gianluca Pirozzi**, M.D., Ph.D., Head of Clinical Development and Translational Sciences  
**Sharon Barr**, Ph.D., Head of Research, Bioinformatics, & Diagnostics |

## Q&A Session II

### 30 minutes

## Conclusion
- **Ludwig Hantson**, Ph.D., CEO
Investor Day Speakers

Alexion Board of Directors & Management Team

David Brennan
Chairman of the Board

Ludwig Hantson, Ph.D.
Chief Executive Officer

Aradhana Sarin, M.D.
Chief Financial Officer

John Orloff, M.D.
Head of R&D

Brian Goff
Chief Commercial & Global Operations Officer

Alexion R&D Leadership & Clinical Experts

Gianluca Pirozzi, M.D., Ph.D.
Head of Clinical Development & Translational Sciences

Cristina Quarta, M.D., Ph.D.
CAEL-101 Clinical Development Lead

Darius Moshfeghi, M.D.
Consultant, ALXN2040 GA & Professor of Ophthalmology, Stanford University

Anita Hill, M.D., Ph.D.
Hematology Global Medical Affairs Lead

Sharon Barr, Ph.D.
Head of Research, Bioinformatics, & Diagnostics
Vision & Strategy

Ludwig Hantsen, Ph.D.
Chief Executive Officer

Aradhana Sarin, M.D.
Chief Financial Officer
Rare Disease By The Numbers

- **30 million people in the United States** are affected by rare disease that is approximately 1 in 10.
- **5%** of all rare diseases are approved treatments available for only 7,000 rare diseases are known to exist today.
- **50%** of rare disease patients in the U.S. are children.
- **7,000** rare diseases are known to exist today.
- **1 in 10** rare disease patients in the U.S. are children.

Source: GAO Orphan Drugs November 2016; Global Genes
Chelsey advocated for herself and was diagnosed with NMOSD in 2019
Our Mission:
Transform the lives of people affected by rare diseases and devastating conditions by continuously innovating and creating meaningful value in all we do.
Committed to Our Focused Rare Disease Model

R&D
Internal complement innovation; committed to maximizing value of platform technologies with reinvigorated focus on speed to proof-of-concept

Commercial
High-touch patient support, innovative diagnostics, patient-centered access models, and experienced rare disease field teams

Financial Execution & Culture
Best-in-class financial discipline with culture focused on quality and compliance
Differentiated as a biotech focused in rare disease with resourcing capacity to reinvest in our pipeline and commercial capabilities; ambition for >5 INDs by 2025

Robust pipeline with terminal complement, Factor D and anti-FcRn Platforms and novel rare disease assets contributing to 7 blockbuster franchises; multiple pivotal results in next 12+ months

Key Takeaways

Sustainable growth in portfolio targeting $9-10B in revenue in 2025\(^1\) (current consensus estimate \(\sim\)$7.5B\(^2\)) while maintaining >50% non-GAAP operating margins

Current pipeline contributes >$10B in peak sales potential beyond 2025

\(^1\) 2025 $9-10B target is at constant currencies (9/30/20 levels); \(^2\) Consensus estimate as of Alexion Investor Day on October 6, 2020
Proven Operational Excellence

Global Organization Continuing to Innovate for Patients

- Established ULTOMIRIS as PNH Standard Of Care Across Major Geographies (US, DE, JP)
- SOLIRIS First Approved Therapy For NMOSD
- Demonstrated Commercial Excellence: Neurology Largest US Franchise After Just 2 Years

Consistent Financial Execution\(^1\)

- Double Digit Revenue Growth ($M)
  - +16% CAGR

- Non-GAAP Margin Expansion +840bps

- Nearly Doubled Non-GAAP EPS

\(^1\)A reconciliation of GAAP to non-GAAP financial results is provided in the appendix and is available at www.alexion.com; *2020E based on midpoint of 2020 Guidance issued July 30, 2020. The financial guidance (and related assumptions) set forth herein was provided as of July 30, 2020 (such guidance has not been updated to reflect any events subsequent to July 30, 2020).
Targeting $9-10B in Global Revenues in 2025

Key Growth Drivers from 2020 to 2025 to Achieve $9-10B in Global Revenues
- Organic SOLIRIS/ULTOMIRIS CS Neurology portfolio growth
- Metabolic (STRENSIQ, KANUMA) volume growth
- ANDEXXA utilization expansion

Operational Execution and Capital Allocation
- Initial revenue contribution from 10 launches by 2023
- Maintain >50% non-GAAP operating margins
- Dedicate at least 1/3 of FCF toward share repurchases

Beyond 2025
- Robust pipeline maximizing existing assets with >$10B+ in peak sales potential
- Continued financial execution and strong FCF generation allows for reinvestment

SHARE BUYBACK PROGRAM (~10% REDUCTION IN O/S) WITHOUT INCREASING LEVERAGE (~1.0X)

1 Ambition Baseline - 12/31/19 1,885 patients  
2 Relative to 12/31/19 share count, excluding impact of new issuances  
3 2025 $9-10B target is at constant currencies (9/30/20 levels)
Our Value Creation Strategy

**LEAD AND EXPAND IN COMPLEMENT**

**LEAD**
- Establish ULTOMIRIS as the new standard of care
  - PNH
  - aHUS
  - Neurology in 2022/2023
- Develop and launch next-generation innovative C5 formulations

**EXPAND**
- Expand presence in Neurology
- Focus new ULTOMIRIS expansion opportunities on direct-to-Phase 3, rapid Proof of Concept

**DIVERSIFY INTO NEW GROWTH AREAS**

**DIVERSIFY**
- Execute novel asset development to expand rare disease focus
- Grow acute care presence with Andexxa launch

Secure and grow our base business

Drive new growth opportunities outside C5
Novel Platforms & Assets Further Diversification

**Novel Platforms**

- **Continuous Innovation in C5 through Transformative Therapies**
  - PhN
  - aHUS
  - ALS
  - NMO
  - ANCA

- **ULTOMIRIS 2nd Gen**
  - PhN
  - aHUS
  - ALS
  - NMO
  - ANCA

- **ALXN17190 3rd Gen**
  - PhN
  - aHUS
  - ALS
  - NMO
  - ANCA

- **Non-C5**
  - **Factor D**
    - ALXN2050 Ph2
    - Geomorphic Atrophy
    - PhN
    - aHUS
  - **Anti- Properdin**
    - ALXN2050 Ph2
    - PhN w/ EVH
    - PhN
    - aHUS
  - **Multiple large, rare disease opportunities**

- **Non Complement**
  - **And Expanding Beyond Complement**
    - ALXN1830
    - Anti-FcRn
    - Ph1
    - gM

**Novel Assets**

- **ALXN1840**
  - Ph3
  - Wilson Disease

- **CAEL-101**
  - Ph3
  - AL-Amyloidosis

- **ANDEXXA**
  - Ph2
  - Urgent Surgery

- **AG10**
  - Ph3
  - ATTR-CM

*Legend for Novel Platforms & Assets*

- **Hematology**
- **Nephrology**
- **Metabolics**
- **Neurology**
- **Cardiology**
- **Ophthalmology**
- **Acute Care**
# Building 7 Blockbuster Franchises

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<tr>
<th>Hematology</th>
<th>Nephrology</th>
<th>Metabolics</th>
<th>Neurology</th>
<th>Cardiology</th>
<th>Ophthalmology</th>
<th>Acute Care</th>
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<tr>
<td>Paroxysmal Nocturnal Hemoglobinuria (PNH) Ultra-Rare&lt;sup&gt;1&lt;/sup&gt;</td>
<td>Atypical Hemolytic Uremic Syndrome (AHUS) Ultra-Rare&lt;sup&gt;1&lt;/sup&gt;</td>
<td>Hypophosphatasia (HPP) Ultra-Rare&lt;sup&gt;1&lt;/sup&gt;</td>
<td>Generalized Myasthenia Gravis (gMG) 60-80K U.S. Only</td>
<td>AL Amyloidosis ~20K U.S. &amp; EU Only</td>
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<td>Warm Autoimmune Hemolytic Anemia (WAIHA) ~65K U.S. and EU</td>
<td>Hematopoietic Stem Cell Transplantation&lt;sup&gt;2&lt;/sup&gt; (HSCT-TMA) ~5K U.S. Only</td>
<td>Lysosomal Acid Lipase Deficiency (LAL-D) Ultra-Rare&lt;sup&gt;1&lt;/sup&gt;</td>
<td>Neuromyelitis Optica Spectrum Disorder (NMOSD) ~10K U.S. Only</td>
<td>Geographic Atrophy (GA) ~2M U.S. and EU</td>
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<td>Complement Mediated TMA (CM-TMA) ~2K Addt US Oppty</td>
<td>Wilson Disease ~10K U.S. and EU</td>
<td>Amyotrophic Lateral Sclerosis (ALS) &gt;40K U.S., EU, &amp; Japan</td>
<td>Transthyretin Amyloid Cardiomyopathy&lt;sup&gt;4&lt;/sup&gt; (ATTR-CM) &lt;6K Japan Only</td>
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<td>Renal Basket (LN, IgAN, PMN, C3G) &gt;200K U.S. Only</td>
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<td>Guillain-Barre Syndrome&lt;sup&gt;3&lt;/sup&gt; (GBS) &lt;2K Japan Only</td>
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Source: Internal epidemiological analysis; <sup>1</sup>Ultra-Rare: <6K; <sup>2</sup>Incident population; <sup>3</sup>Currently pursuing Japanese development only; <sup>4</sup>ALXN has rights to Japanese development only
Development-Stage Pipeline with >$10B+ in Potential Peak Sales

LEAD and EXPAND in complement

- ULTOMIRIS
  - New Indications
    - ALS (2023)
    - HSCT-TMA (2023)
    - CM-TMA (2023)
  - $1 to $4B

- ALXN1720
  - DM (2023+)
  - $2 to $3B

- ALXN1840
  - Wilson Disease (2022)
  - $500M to $1B

- ANDEXXA
  - Factor Xa (2020)
  - Urgent Surgery (2023+)
  - $1.0B+

- CAEL-101
  - AL Amyloidosis (2023)
  - ATTR-CM (2023)
  - Japan Only
  - $1.0B+

- ALXN1830
  - WAIHA (2023+)
  - gMG (2023+)
  - $1.0B+

DIVERSIFY into new growth areas (sourced through BD)

- ALXN1830
  - WAIHA (2023+)
  - gMG (2023+)
  - $500M to $2.0B+

- Factor D
  - PNH (2023+)
  - GA (2023+)
  - Renal (2023+)
  - $1.0B+

7 Blockbuster Franchises

- Hematology
- Nephrology
- Metabolics
- Neurology
- Cardiology
- Ophthalmology
- Acute Care

Illustrative only; timing shown represents launch year; based on non-adjusted peak revenue estimates for incremental market opportunity; 1Structured as an option to acquire Caelum; 2Factor D represents both ALXN2040 and ALXN2050
Board of Director Perspectives

David Brennan, Chairman of the Board

VALUE

GROWTH
R&D Portfolio Overview

John Orloff, M.D.
Head of R&D
Advancing a Differentiated Rare Disease Pipeline

Leading R&D Engine Supports Rare Disease Innovation

- Global regulatory team with deep rare disease expertise
- High-quality, fit-for-purpose PK/PD modeling and simulation
- Robust clinical operations capabilities leveraging Artificial Intelligence (AI) in clinical trial development
- Diverse clinical development expertise across therapeutic areas to fully leverage potential of platform technologies
- Deep complement biology supporting our internal innovation engine to unlock potential throughout complement cascade
- Patient-centered model informs clinical trial design

ROBUST PIPELINE DRIVING NUMEROUS UPCOMING CATALYSTS

**2021**
- ALXN1840 Wilson Ph3 TLR* (1H)
- ULTOMIRIS COVID-19 Ph3 TLR (1H)
- ALXN2050 PNH Ph2 TLR (2H)
- ULTOMIRIS gMG Ph3 TLR (2H)

**2022**
- ULTOMIRIS NMOSD TLR (1H)
- ULTOMIRIS SC PNH/aHUS launch (mid-22)
- AG10 Japan Ph3 TLR (2H)
- ULTOMIRIS ALS Ph3 TLR (2H)
- CAEL-101 Ph3 TLR (2H)
- ALXN1840 Wilson US launch (2H)
- ULTOMIRIS gMG US launch (2H)

UNIQUE CAPABILITIES DRIVE ROBUST PIPELINE WITH MULTIPLE POTENTIAL BLOCKBUSTERS

*TLR: Top line readout
Targeting 10 Launches by 2023

**LEAD AND EXPAND IN COMPLEMENT**

- **Ph3 Trial To File 3Q ’21**
  - PNH / aHUS
  - U.S. Ultra-Rare Population
  - First in Class
  - SC Infusion
  - CS Inhibitor
  - ULOMIRIS
  - gMG
  - U.S. Diagnosed Population
  - ~20K
  - ~15K

- **Ph3 Trial To Initiate 4Q ’20**
  - U.S. Target Population
  - ~4.5K
  - First in Class
  - CS Inhibitor
  - ULOMIRIS
  - ALS

- **Ph3 Trial To Initiate 1H ’21**
  - ~2K
  - Add U.S. Opportunity
  - Best in Class
  - CS Inhibitor
  - SOLIRIS
  - NMOSD

- **Ph3 Trial Initiated 3Q ’20**
  - ~10K
  - JP Diagnosed Population
  - First and Only
  - Targeted Therapy
  - CM-TMA

- **Ph3 Trial Initiated 1H ’21**
  - <2K
  - JP Diagnosed Population
  - Best in Class
  - CS Inhibitor
  - SOLIRIS
  - GBS

- **Ph3 Trial On-Going; Data 1H ’21**
  - ~5K
  - Potential Superiority vs Standard of Care
  - Best in Class
  - Small Molecule
  - ATTR-CM

- **Ph3 Trial To Initiate 4Q ’20**
  - ~6K
  - U.S. PNH Population
  - First and Only
  - Targeted Therapy
  - Wilson Disease

- **Ph3 Trial To File 3Q ’21**
  - ~<6K Each
  - <10%
  - U.S. PNH Population
  - First and Only
  - Targeted Therapy
  - PNH with EVH

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# Robust Clinical Stage Pipeline Progress since 2017

## SOLIRIS
- IV (GBS) Japan Only
- IV (NMO/SD)
- IV (PNH)
- SC Weekly
- SC FcRn (WAIHA)
- IV (gMG)
- IV (CMS-TMA)
- IV (CM-TMA)
- IV (TMA)
- IV (Renal Basket)
- IV (COVID-19)
- SC (DM)

## ULTOMIRIS
- IV (ALS)
- IV (CM-TMA)
- IV (Renal Basket)
- IV (COVID-19)
- SC (gMG)
- SC (DM)
- Oral (Wilson)
- Oral (WAIHA)
- Oral (PNH with EVH)
- Oral (ATTR-CM) Japan Only
- Oral (PNH Monotherapy)
- Oral (Renal Basket)
- ANDEXXA-S (Urgent Surgery)

## ALXN1720
- Oral (PNH Monotherapy)
- Oral (Renal Basket)

## DIVERSIFY
- ANDEXXA
- ANDEXXA
- ANDEXXA-S
- CERDULATINIB
- Lymphoma (CTCL, PTCL, FL)

## Timeline
- **END OF 2017**
  - Ph3
  - Ph3
  - Ph3
  - IV (PNH)
  - SC Weekly
- **2020 PHASE 1**
  - Ph3 to Initiate 1H ‘21
  - Ph3 to Initiate 3Q ‘20
  - Ph2 to Initiate 2H ‘21
  - Ph2 to Initiate 3Q ‘20
- **2020 PHASE 2**
  - Ph3 to Initiate 1H ‘21
  - Ph3 to Initiate 1H ‘21
  - Ph3 to Initiate 2H ‘21
  - Ph2 to Initiate 1H ‘21
  - Estimated TLR 2H ‘21
- **2020 PHASE 3**
  - Approved
  - Approved
  - Estimated TLR 2H ‘21
  - Estimated TLR 2H ‘22
  - Estimated TLR 1H ‘21
- **2020**
  - PHASE 1
  - PHASE 2
  - PHASE 3

## Notes
1. TLR: Topline readout; 2. Adults with COVID-19 who are hospitalized with severe pneumonia or acute respiratory distress syndrome (ARDS); 3. T20 currently in HV Ph1 with topline readout estimated 1H ‘21 and subsequent DM and gMG trials to begin after that; 4. 1830 Ph1 HV program to reinitiate for SC formulation with WAIHA and gMG Ph2 programs to follow in 2021; 5. Structured as option to acquire Caelum; 6. Exclusive license to develop & commercialize in Japan.
Lead & Expand: Sustainability in our C5 Business

Brian Goff
Chief Commercial & Global Operations Officer

John Orloff, M.D.
Head of R&D
Commercial Model Tailored to Rare Disease

**Rare Disease Focused Field Force**
Increasing depth of experience building physician education and KOL relationships
- Specialized therapeutic area expertise
- Extensive tenure in pharma / biotech

**Best In Class Data Analytics**
Innovating to find alternative ways to help a rare disease patient receive their diagnosis earlier
- Artificial intelligence using de-identified claims to ID potential HCPs who may have rare disease patients

**Operational Excellence**
High quality, secure, and consistent manufacturing and supply
- Comprehensive global footprint
- Robust distribution & supply chain

**Patient-centered Access Models**
Building sustainable pricing solutions to support patient access to treatments
- ULTOMIRIS pricing strategy
- HEOR, Access, Contracting Field Support

**Personalized High-Touch Patient Support Services**
Simple and continuously improving experience for patients/caregivers to support patients initiating and maintaining access to treatment
- Individual Case Managers
- Vaccination Support
- Funding Assistance
- Home Infusion Assistance

"OneSource is a U.S. Patient Service Model"
Innovating to Remain at the Forefront of C5 Science

**First generation C5**
- Ultra-Rare Focus
- <6K Patients

**Second generation C5**
- Expanding to Rare
- >50K Potential Addressable Patients

**Third generation C5**
- Continued Expansion in Rare
- >100K Potential Addressable Patients

**ULTOMIRIS 100mg/mL**
- High Concentration
- Delivers on patient preference for shorter infusion time (45 minutes)
- Planned 2020 launch

**ULTOMIRIS**
- Q8W IV
- Annual treatment cost per patient significantly lower than SOLIRIS\(^2\)
  - 10% in PNH
  - 33% in aHUS, gMG, NMOSD

**ULTOMIRIS Subcutaneous**
- Once-weekly, 10 min self-administration
- On-body commercially available device
- Planned 2022 launch

**ALXN1720**
- Subcutaneous Mini-body
- Potential auto-injector or pre-filled syringe administration
- Plan to target broader rare diseases with burdensome, chronic standard of care

1US estimated launch; pending FDA approval; 2On-going treatment cost after first year of treatment.
Neurology is Key Growth Driver through 2025

Significant Progress Thus Far Against 4X Neuro Ambition

- Jan 2020: Ambition to expand U.S. treated population 4x by 2025

- 4Q 2017: gMG Launch
- 2Q 2019: NMOSD Launch
- Jun 2020: 2,341 Total Patients on SOLIRIS

Oppotunity to Expand Neurology Presence with ULTOMIRIS in gMG & NMOSD

*Ambition Baseline - 12/31/19 1,885 patients (4x growth ambition includes only gMG and NMOSD indications); 2,341 net patients on therapy as of Jun 30, 2020
ALS is a Devastating, Fatal Disease

**Amyotrophic Lateral Sclerosis (ALS)**
- Progressive loss of upper and lower motor neurons that inhibits signals
- Muscles begin to atrophy leading to loss of speech, paralysis, respiratory failure, and death
- Delayed time to diagnosis with average of 9-15 months from first symptom onset
- Average survival time is 3-5 years

**Significant Unmet Need**
- Available treatment options not effective
- Need for new treatment options with potential to halt or significantly slow disease progression and demonstrate survival and functional benefit
- Improved education and faster path to diagnosis

**Estimated Addressable Population**
- **>40K**: estimated diagnosed patients in US, EU, and JP
- **~75%**: as 25% of patients may have late stage disease and may not benefit from treatment
- **~30K**: addressable patient population with potential to benefit from ULTOMIRIS

**ALS Symptomology**
- Head and neck symptoms:
  - Impaired speech
  - Excess saliva
  - Difficulty swallowing
- Upper body symptoms:
  - Hand weakness
  - Limited range of motion
  - Upper body muscle spasms
  - Trouble with dressing/hygiene
  - Impaired handwriting
  - Difficulty preparing food
- Respiratory symptoms:
  - Shortness of breath
  - Restricted breathing
  - Difficulty sleeping
- Lower body symptoms:
  - Frequent tripping
  - Difficulty on stairs
  - Weak feet
**Role of Terminal Complement (C5) Inhibition in ALS**

**ULTOMIRIS Phase 3 ALS Trial Represents our Commitment to Pursue Promising Science**

- Role of C5 inhibition in CNS and neuromuscular junction confirmed with SOLIRIS approval in gMG and NMOSD
- Preclinical evidence supports pursuit of ULTOMIRIS Phase 3 trial in ALS
  - Survival benefit shown in SOD1 mouse models through C5a inhibition or C5aR1 genetic knock-out
  - ALS patient tissue analysis shows elevated C5a levels in blood and lymphocytes; increased C5b-9 activity observed at neuromuscular junction prior to nerve death

ULTOMIRIS ALS Phase 3 Trial Design

**Disease Overview**

- Potential to halt disease progression and symptom improvement
- Biomarker collection enhances evidence generation of role of C5 in ALS
- Development program built on patient and KOL insights which is critical in building a compelling value proposition

**Mechanism of Action**

**Clinical Development**

- Top-line Results Anticipated 2H 2022
- 2:1 Randomization
- Randomized controlled 50 weeks
- Open-label Extension 106 weeks

- Screening Up to 4 weeks (N=354)
- Enrolling familial & sporadic ALS patients with onset <36 months from screening

- Primary Endpoint: ALS-FRS-R
- Key Secondary Endpoints: Ventilator-assisted free survival (VAFS), Slow Vital Capacity (SVC), Muscle strength (Hand-held dynamometry - HHD)

**Potential ULTOMIRIS Value Proposition**

ULTOMIRIS HAS THE POTENTIAL TO BE FIRST-IN-CLASS C5 INHIBITOR IN ALS
ALXN1720: Internally Developed Third Generation C5 Inhibitor

**Indication Selection: gMG**

- 25 kDa molecular weight (most antibodies ~150 kDa)
- Potential for auto-injector or pre-filled syringe
- Extended half-life by binding to human serum albumin

**ALXN1720: Bi-Specific Mini-body**

- Long-acting, small volume SC dosing

**PHASE 1 TOP LINE DATA AND SUBSEQUENT PHASE 2 STUDY STARTS EXPECTED 1H 2021**

**ALXN1720 Asset Overview**

**Indication Selection: DM**

- Small-volume, once-weekly dosing

**Expanding: Ultomiris in ALS & ALXN1720**
Evolving our gMG Portfolio Strategy with ALXN1720

Expanding Addressable gMG Patient Population with ALXN1720

Ability with **ALXN1720** to treat earlier-line patients
- Mild, moderate & severe AChR+ patients
- Subcutaneous once-weekly dosing
- Auto-injector or pre-filled syringe
- Can co-administer with IVIg or anti-FcRn

**ULTOMIRIS** expands addressable patient population
- Moderate-to-severe AChR+ patients
- No prior treatment failure requirement
- ~30% discounted annual treatment cost per patient

**SOLIRIS** first approved gMG treatment in 60 years
- Refractory AChR+ patients who failed prior therapies

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1AChR+: Acetylcholine receptor antibody-positive gMG represents ~85% of total gMG patients
**Dermatomyositis (DM)** is a rare autoimmune inflammatory myopathy characterized by chronic inflammation and degeneration of muscle and skin.

### Classic DM: Muscular and Cutaneous Manifestations

**Cutaneous:** Visible Symptoms Often Support Diagnosis
- Lesions on hands
- Heliotrope rash
- Cotton’s papules

**Muscular:** Impact on Patient QoL Driven by Muscle Weakness
- Progressive muscle weakness that worsens over time
- Often involves hips, thighs, shoulders, upper arms, neck
- Everyday tasks (holding objects, rising from chair, climbing steps) are difficult
- Difficulty swallowing, breathing problems
- Despite therapy, ~1/3 of patients are left with mild to severe disability

- **With SoC therapy, 5-year mortality is ~25%**
- **Patients' cancer risk is elevated**

### Role of Complement in DM
- Classical and alternative pathway proteins are upregulated in muscle of dermatomyositis (DM) patients
- Terminal complement (C5b-9) deposition on non-necrotic and necrotic muscle fibers
- Eculizumab improved clinical condition and biological parameters in a juvenile patient at 900 mg QWx5 - >1200mg Q2W

### DM Diagnosed Prevalence
- ~50K
- Classic DM Diagnosed Prevalence ~40K

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ALXN1840 in Wilson Disease

John Orloff, M.D.
Head of R&D

Brian Goff
Chief Commercial & Global Operations Officer
Wilson Disease is a Rare Inherited Disorder

- Devastating disorder characterized by copper accumulation in the liver, brain, and other organs
- If left untreated, risk of eventual cirrhosis or liver failure
- Need unaddressed by current chelators:
  - De-coppering and elimination through normal excretion
  - Poor compliance
  - Potential for neurological worsening

**Diagnosed Prevalence**

- USA ~5K
- EU ~5K
- Japan ~2.5K

*Genetic prevalence believed to be higher and potential to improve for some patients with increased awareness and new diagnostic practices*

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**Significant Unmet Need**

**Challenging Path To Diagnosis**
- Heterogeneous symptomology
- Complex diagnostic algorithm
- High rate of mis-diagnosis

**Poor Compliance Rate with Standard of Care (SoC)**
- Current SoC consists of cycles of de-coppering agents followed by zinc maintenance
- Burdensome treatment regimen and side-effects
  - 2-4x daily oral requiring 6-12 hrs fasting per day

**Neurological Worsening**
- Long-term neurological effects include impaired motor skills and psychiatric changes (behavior and personality)
- Current SoC does not address neurological impacts of disorder
Our Patients are Our Inspiration: Meet Cory
DIVERSIFICATION OPPORTUNITY: ALXN1840 IN WILSON DISEASE

ALXN1840: Novel Oral Potential Treatment for Wilson Disease

**Wilson Disease**
- Disease Overview
- Patient Journey
- Mechanism of Action
- Clinical Development
- Path to Market

**Tripartite Complex**
- ALX
- ALXN1840
- PT
- Protein
- CU
- Copper

1. **ALXN1840 dosing regimen:** *Oral, once-daily dose*
2. **High affinity to copper (10,000x): direct binding and removal of copper from intracellular stores** in the liver
3. **ALXN 1840 specifically binds to copper**
4. **ALXN1840 + copper forms stable tripartite complex with proteins**
5. **Normal biliary excretion of copper**

---

New Biomarker May Change Wilson Disease Diagnostic Paradigm

Alexion is developing a new biomarker designed to directly quantify the labile bound copper (LBC) fraction.

- LBC assay for screening, diagnosing, and assessing copper control in patients
- Expected to allow clinicians to easily and accurately assess patients
- Establish reliable treatment targets that directly align with ALXN 1840 treatment goals
- Suitable for the analysis of all pre- and post-dose samples, regardless of treatment
New Phase 2 Data Suggests Potential To Redefine Treatment Goals

Recent Phase II data shows that ALXN1840 actively and dynamically mobilizes Cu from tissue stores.

Treatment Naïve
- Rapid mobilization of copper from tissues
- Sustained copper elimination

Treatment Experienced
- Mobilization of stores from incomplete tissue decoppering
- Sustained copper elimination

Based Upon Ph2 Data, Exploring A Revised Endpoint To Support:

Superiority of ALXN1840 over SOC
Potential paradigm shift in treatment from plasma copper guided to whole body copper mobilization and excretion approach
Global access & value strategy

Additional Programs Designed to Support Broader Decoppering:

Copper Balance Program
- Measure copper mobilization & elimination through biliary system

Liver Biopsy Program
- Measure liver tissue de-coppering
Commercial Plan to Bring ALXN1840 to Patients

Key Objectives For Launch

1. Drive Education, Awareness & Diagnosis
   - Increase awareness amongst key physicians
   - Develop early screening tools
   - Introduce LBC Assay to improve screening, diagnosis, and assessment of copper control in patients
   - Increase awareness that early diagnosis/treatment can prevent often irreversible psychiatric impacts

2. Revolutionize the Treatment Paradigm
   - ALXN1840 is a first-in-class agent that binds copper with high affinity and specificity
   - ALXN1840 has a unique mechanism (proven in Ph2) for copper control
     • Rapid, sustained control of copper and clinical symptoms with low risk of neurological worsening

3. Drive Access & Conversion from Current Treatments
   - Leverage potential strength in clinical profile and convenience of once-daily oral administration
   - Mobilize health economics and access resources to support labeling, access, and pricing focused on superiority (based on potential Ph3 results)
CAEL-101 in AL Amyloidosis

Cristina Quarta, M.D., Ph.D.
CAEL-101 Clinical Development Lead

- More than 15 years of experience specializing in field of systemic amyloidosis in major international amyloid centers
- Published over 50 manuscripts including global guidelines on diagnosis and management of cardiac amyloidosis
- Joined Alexion in 2019
AL-Amyloidosis: Hematological, Severe, Multi-Organ Disorder

Disease Overview

A plasma-cell dyscrasia characterized by an autonomous proliferation of plasma cells with an overproduction of a monoclonal IgG

Severe multi-organ damage most frequently heart, kidneys, and peripheral nerves

Difficult to recognize because of its broad range of manifestations and vague symptoms, and lack of awareness

Clinical Data

Primary Treatment Goals

1. Suppression of amyloid protein precursors
2. Reduction of amyloid deposits in the organs
3. Symptom relief, survival & organ function improvement

Unmet Medical Need

Median Survival <18 Months from Diagnosis

Addressable Population

~20K US + EU Addressable Population

(Mayo Stage IIIa + b)
Early Data Supports Role of CAEL-101 in AL-Amyloidosis

CAEL-101 is a chimeric mAb with high specificity to kappa and lambda light chain antibodies

Phase 1a/1b trial was first in AL-Amyloidosis to show improvement in cardiac function via GLS

*Global Longitudinal Score (GLS) is a better predictor of survival vs. cardiac biomarkers*

Long-term Phase 1a/1b data show 78% survival (15/19) at median follow-up of more than 3 years (37 months) in patients treated with CAEL-101

Left ventricular data: Phase 1b Cardiac Patients (N=10)

Mean absolute improvement of 1.69 points in GLS score after 12 weeks of treatment

- Evidence suggests patients treated with chemotherapeutic PCD agents showed no change in GLS from baseline to at least 1 year
- 9 of 10 cardiac patients improved compared to baseline
- CAEL-101 was well-tolerated, no dose-limiting toxicity or drug-related deaths; maximum tolerated dose 500mg/mg²


Bhutani, et al. Long term follow-up of patients with AL Amyloidosis treated on a phase 1 study of Anti-Amyloid Monoclonal Antibody CAEL-101
Transforming AL-Amyloidosis Treatment Landscape

CAEL-101 Phase 2/3 Program Design

CAEL-101 has potential to remove amyloid deposits from tissues and improve organ function, with aim of prolonging survival

With Potential to Change Standard of Care

Current

Amyloid Suppression: CyBorD

Amyloid Removal: CAEL-101

Future

Amyloid Suppression: CyBorD + Daratumumab

Primary Endpoint: Overall Survival

Secondary Endpoints: Patient Function (6MWT), QoL (KCCQ-OS and SF-36v2 PCS), and Cardiac Imaging (GLS%)

CAEL-101 to be studied alongside plasma cell dyscrasia (PCD) treatment

DIVERSIFICATION OPPORTUNITY: CAEL-101 IN AL AMYLOIDOSIS

ON TRACK FOR POTENTIAL LAUNCH BY 2023; OPTION TO ACQUIRE CAELUM POST PHASE 3 DATA
Factor D Platform

Gianluca Pirozzi, M.D., Ph.D.
Head of Clinical Development and Translational Sciences

- Over two decades of drug development experience
- Previously served as a Board Member of Imbria Pharmaceuticals and current Board Member of Timber Pharmaceuticals
- Scientific Advisor for the Smith-Magenis-Syndrome Research Foundation
- Joined Alexion in 2019
Targeting Factor D to Inhibit Alternative Pathway

- Factor D is a key, rate-limiting enzyme of the complement alternative pathway.
- Selective inhibition of the alternative pathway keeps classical and lectin pathways intact to fight infections.
- Proof of Concept shown for Factor D inhibition in multiple complement-mediated rare diseases.
- More likely to maintain consistent control than drugs targeting C3 or Factor B.
  - C3 and fB are acute phase reactants (increasing levels during inflammation or stress).

### Complement Cascade

- **Classical Pathway**
- **Lectin Pathway**
- **Alternative Pathway**

### Factor D an Attractive Target for AP Inhibition

More tractable therapeutic target vs Factor B given much lower concentration in blood.

<table>
<thead>
<tr>
<th>Protein</th>
<th>Concentration</th>
</tr>
</thead>
<tbody>
<tr>
<td>C3</td>
<td>6.49 μM</td>
</tr>
<tr>
<td>Factor B</td>
<td>2.15 μM</td>
</tr>
<tr>
<td>Factor D</td>
<td>0.08 μM</td>
</tr>
</tbody>
</table>

Relative circulating concentration of complement target proteins.
Factor D Platform

Two Unique Assets Targeting Factor D

**ALXN2040**
- Optimal biodistribution in the retina, supports potential use in ophthalmology indications

**ALXN2050**
- Longer half-life and more potent alternative pathway inhibition supports potential use in PNH monotherapy and renal disease

Wide Range of Therapeutic Areas of Interest

- Neurology
- Dermatology
- Pulmonology
- Nephrology
- Gastroenterology

Multiple Ascending Dose Results

- AP = alternative pathway; danicopan = ALXN2040; ACH-5528 = ALXN2050
- >95% AP inhibition at mean steady state trough concentrations in P1 MAD study in healthy volunteers as measured by AP Hemolysis and AP Wieslab assays
Anita Hill, M.D., Ph.D.
Hematology Global Medical Affairs Lead

- Prior Lead Clinician for the National PNH Service, UK and Honorary Associate Professor for the University of Leeds
- Managed over 500 patients with PNH in her career
- Joined Alexion in February 2020

Darius Moshfeghi, M.D
Professor, Stanford University & GA Program Clinical Consultant

- Professor of Ophthalmology, Stanford University School of Medicine
- Chief of Retina Division, Byers Eye Institute
- Joined Alexion in 2020 as clinical consultant for GA
Alexion Continues to Innovate for Patients with PNH

Patients managed with supportive care
20-35% died within 6 years of diagnosis

First approved treatment in PNH
Improved overall survival to similar level of general population

First and only long-acting C5 inhibitor
Maximal IVH control and reduction in BTH, while reducing treatment burden

Enhancing Treatment Offering
Transfusion dependent EVH and additional patient choice to further reduce treatment burden

Management and Care of PNH Patients in Practice

Commencement of C5 Inhibitor Treatment

LDH?
Reduced
Unchanged
Assess for C5 Mutation

≤ 1.5 x ULN
Hgb?
Satisfactory Response
Symptomatic Anemia

> 1.5 x ULN
Elevated
Low / Normal

Reasons for Continued Transfusions
- Bone Marrow Failure
- Folate Deficiency
- Relative EPO Deficiency
- Iron Overload
- Alloantibodies
- Breakthrough Intravascular Hemolysis
- Extravascular Hemolysis
- Hypersplenism
- Bleeding

~10% of PNH Patients Suffer from Transfusion Dependent EVH

1BTH: breakthrough intravascular hemolysis; 2EVH: Extravascular hemolysis; 3Hgb: Hemoglobin; 4Retics: Reticulocytes; 5ULN: Upper limit of normal; 6EPO: Erythropoietin
Progressing Factor D Development Plans in PNH

**Phase 3 ALXN2040 Combination Study**

- **Objectives:**
  - Address clinically evident EVH for patients on ULTOMIRIS or SOLIRIS (<10% of patients) with 2040

- **Primary Endpoint:** Change from Baseline in hemoglobin

- **Study Details:**
  - 2:1 Randomization of ULTOMIRIS or SOLIRIS + ALXN2040
  - (N=84) for 12 weeks
  - Patients will continue their C5 regimen throughout study duration to ensure continued IVH control

**Phase 2 ALXN2050 Study**

- **Objectives:**
  - Potential Transformational Oral Therapy
  - Address IVH with no C3 deposition leading to EVH

- **Study Details:**
  - (N=26) for 12 weeks
  - LT Extension
  - Patients receiving ALXN2040 monotherapy

**Targeting Factor D In PNH**

**ALXN2040:**
- **Combination Therapy To Address EVH**
  - Potential to address clinically evident EVH for patients on ULTOMIRIS or SOLIRIS (<10% of patients) with 2040
  - Primary Endpoint: Change from Baseline in hemoglobin

**ALXN2050:**
- **Potential Transformational Oral Therapy**
  - Ability to build on fD platform through 2050 and address IVH with no C3 deposition leading to EVH
  - Continue to ease treatment burden on patients & healthcare system through an oral administration only
  - Reduce burden on healthcare systems & resources
Geographic Atrophy & Broader AMD Landscape

Age-Related Macular Degeneration (AMD)

- **Early**
  - 5% will develop in 10 years (14% in 10 years if other eye has AMD)

- **Intermediate**
  - No approved treatments.
  - AREDS vitamins and behavioral modification

- **Advanced**
  - No treatments

- **Wet AMD**

Geographic Atrophy (GA)

- Chronic and progressive degeneration of the portion of the retina responsible for central & color vision
- Slow rate of progression and subtle symptoms leads to lower rates of diagnosis than wet AMD
  - One affected eye typically impacted first
  - Disproportionately affects those 75 and older
- Affects >2M patients across US and EU
  - 40% of whom are diagnosed and addressable
  - Growing 3-5% per year as population ages

High Disease Burden for Patients

- Major cause of blindness
- High levels of visual impairment and loss of mobility
- Significant reductions in quality of life

Geographic Atrophy

- **Non-Central GA** (does not involve fovea)
  - 80% to 85% of patients at initial diagnosis
  - Difficult to detect if other eye has normal vision

- **Central GA** (involves fovea)
  - 15% to 20% of patients at initial diagnosis
  - Patients may only retain eccentric gaze
Role of Complement and AP Inhibition in Geographic Atrophy

Complement Inhibition Proven Role In GA

- Human studies demonstrate evidence of AP complement activation
  - Complement byproducts in donor eye and plasma/serum samples
- Studies show complement genetic factors implicated in risk factors for AMD, including Factor D
- Intravitreally injected complement inhibitors have demonstrated PoC in Phase 2 studies

ALXN2040 Preclinical Data

Two unique traits of ALXN2040 lead to higher and sustained exposure of the drug in choroid, RPE and retina after oral dosing

- Binds to melanin
- Able to pass blood-retina-barrier
- Potential QD dosing

Promising ALXN2040 PK data in Dutch belted rabbit model
ALXN2040 in Geographic Atrophy

Aim To Reduce Disease Progression And Preserve Vision In Patients At Risk For Permanent Vision Loss

- Oral dosing vs intravitreal injections
  - Leading to improved patient acceptance
  - Systemic approach with advantage of treating both eyes simultaneously
- Improve QoL for patients and ability to perform activities of daily living and maintain independence
- Delivers opportunity to intervene earlier in disease for some patients
- Reduce secondary complications due to GA
- Potential for significant health economic advantages versus intravitreally injected therapies

ALXN2040 is uniquely positioned to address the unmet need in GA based on its ability to cross the retina-blood-barrier and its sequestration in ophthalmic tissue

Study to Initiate 2H 2021
Alternative Pathway Inhibition in Rare Renal Diseases

The Alternative Pathway (AP) Is A Strong Mediator Of Abnormal Immune Response Across Several Renal Diseases

- Alternative Complement Pathway activation drives inflammation and an aberrant immune response in the kidney
- Complement target therapy has potential to treat underlying pathophysiology and improve clinical outcomes
- Goal to reduce occurrence end stage renal disease, need for transplant or dialysis, and mortality

Sampling of Potential Basket Trial Indications

<table>
<thead>
<tr>
<th>Disease</th>
<th>Prevalence</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lupus Nephritis (LN)</td>
<td>~60K US</td>
</tr>
<tr>
<td>IgA Nephropathy (IgAN)</td>
<td>~90K US</td>
</tr>
<tr>
<td>Primary Membranous Nephropathy (PMN)</td>
<td>~80K US</td>
</tr>
<tr>
<td>Complement 3 Glomerulopathy (C3G)</td>
<td>&lt;6K US</td>
</tr>
</tbody>
</table>

- Despite treatment, up to ~30% develop end stage renal disease under current SOC
- Approx. 40% of IgAN patients progress to end stage renal disease
- Greatest unmet need is control of proteinuria
- 10-20% of patients progress to end stage renal disease under current SOC
- Median time to end stage renal disease is ~10 years
- In pts who receive kidney transplant, ~70% experience relapse

Significant Potential Value Exists In Nephrology

- High unmet need, particularly in more severe or refractory populations
- High enthusiasm for novel MOAs and approaches; treatments with noteworthy incremental benefit
- Recent developments in the regulatory environment have clarified and expedited development pathways
- POC/Basket approach with Factor D and C5 de-risks the portfolio and allows for nimble decision making as development progresses and treatment landscape evolves

Planned Ph2 Poc Trials 1H2021

POTENTIAL PREVALENCE OF >200K PATIENTS; ENORMOUS UNMET MEDICAL NEED
Anti-FcRn Platform

Gianluca Pirozzi, M.D., Ph.D.
Head of Clinical Development and Translational Sciences
**Anti-FcRn: Targeting Numerous IgG-Mediated Diseases**

- **Broad applicability** across numerous rare IgG-mediated autoimmune diseases
- **Product differentiation** expected to come from efficacy, IgG lowering effect, and convenience of administration
- **Long-term safety profile** to be demonstrated with perceived differences across assets (albumin reductions & headaches)

### Warm Autoimmune Hemolytic Anemia (WAIHA)

**Rare disease driven by presence of pathogenic IgG autoantibodies that trigger hemolysis at normal body temperature**

- Patients face extreme weakness and fatigue; Inherent risk of thrombotic events
- Currently no approved therapies. Most common treatments include steroids, rituximab, splenectomy
- ~65K diagnosed prevalent patients in the US and EU

### Generalized Myasthenia Gravis (gMG)

**Rare chronic, autoimmune, neuromuscular disease that can cause progressive muscle weakness and disability**

- Patients face drooping eyelids, blurry or double vision, slurred speech, difficulty swallowing or choking, shortness of breadth, weakness in limbs that can be debilitating
- Expanding Alexion’s portfolio of treatment options for gMG patients in need
- ~60-80K total gMG patients in the US; targeting mild to moderate patients
Pursuing Path Forward with Subcutaneous Formulation

Positive Early Signal from SC Phase 1 Study

- SC single doses suggest meaningful IgG-lowering potential prior to study pause due to COVID-19
- Preliminary PK/PD modeling suggests 1500mg weekly SC may have the potential to provide >70% IgG lowering
- Dosing would be compatible with convenient SC delivery via on-body device

ALXN1830 Value Proposition

- Superior dosing profile with once weekly subcutaneous administration
- High specificity to IgG
- No effect on albumin, eliminating safety concerns of hypoalbuminemia; no headache seen thus far in SC HV
- Rapid onset of action and sustained IgG lowering after a single dose

ALXN1830 FcRn Landscape

Clinical Development

P L A N  T O  R E - I N I T I A T E  S C  F O R M U L A T I O N  D E V E L O P M E N T  P L A N S  E A R L Y  I N  2 0 2 1
Sharon Barr, Ph.D.  
Head of Research, Bioinformatics & Diagnostics

- Responsible for drug discovery and early development
- Over 15 years of industry experience in precision medicine
- Serves on the board of the Alexion Charitable Foundation
- Joined Alexion in 2013
Enhancing our Foundational Complement Research Platform

Building New Capabilities

Collaborations, Partnerships & Acquisitions

- fD small molecule engineering (Achillion)
- C6 Targets (Complement Pharma)
- RNAi Technology (Dicerna)
- Peptide technology (Zealand)

Expertise in Ab Engineering

C5-targeted Continuous Innovation

- ULTOMIRIS Q8W mAb IV / SC
- ALXN1720 Bi-specific Mini-body
- ALXN1820 Bi-specific Anti-properdin

AMBITION TO FILE >5 NOVEL INDS BY 2025
Robust Early Stage Pipeline

**Internal Discovery**
- **ALXN1820**: SC (Anti-Properdin Bi-specific)
- **ALXN1850**: Next generation asfotase alfa
- **ALXN***: Undisclosed Internal Asset
- **ALXN***: Undisclosed Internal Asset
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- **ALXN***: Undisclosed Internal Asset

**Collaborations**
- **Dicerna**: RNAi Complement Target
- **Zealand**: Peptide Complement Target
- **Complement Pharma**: C6

**Preclinical Development**
- **ALXN1820**: IND Planned 2020
- **ALXN1850**: IND Planned 2020

**PHASE 1**

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**STATE OF THE ART ANTIBODY ENGINEERING PLATFORM & NEW TECHNOLOGY COLLABORATIONS**
Internally Designed Novel Assets: ALXN1820 & ALXN1850

**ALXN1820 – Bispecific anti-properdin**

- Blocks properdin, binds to albumin, extending half life
  - Anti-properdin mechanism of action has shown high C3 inhibition without affecting bacterial opsonization (lower infection risk)
- In-vivo studies indicate well-tolerated safety
- Weekly self-administered subcutaneous dosing
- Potential therapeutic area applications include hematology, pulmonology, nephrology, and dermatology

**ALXN1850 – Next generation asfotase alfa**

- Next generation alkaline phosphatase enzyme replacement therapy for hypophosphatasia
  - Engineered catalytic domain has higher activity vs. natural substrates than asfotase alfa
- In-vivo studies demonstrate potential for enhanced efficacy and well-tolerated safety; high in-vivo exposure
- Projected in-human dose significant reduction from current STRENSIQ dose and number of weekly doses
  - Suitable for weekly self-administrated subcutaneous dosing
  - Potential for expansion beyond pediatric onset HPP

**Planned IND for both 1820 and 1850 in the second half of 2020**
Q&A Session II

30 minutes
CEO Concluding Remarks

Ludwig Hantsch, Ph.D.
Chief Executive Officer
Key Takeaways

- Sustainable growth in portfolio targeting $9-10B in revenue in 2025\(^1\) (current consensus estimate ~$7.5B\(^2\)) while maintaining >50% non-GAAP operating margins

- Current pipeline contributes >$10B in peak sales potential beyond 2025

- Differentiated as a biotech focused in rare disease with resourcing capacity to reinvest in our pipeline and commercial capabilities; ambition for >5 INDs by 2025

- Robust pipeline with terminal complement, Factor D and anti-FcRn Platforms and novel rare disease assets contributing to 7 blockbuster franchises; multiple pivotal results in next 12+ months

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\(^1\) 2025 $9-10B target is at constant currencies (9/30/20 levels); \(^2\) Consensus estimate as of Alexion Investor Day on October 6, 2020
Sustainable Business Growing at >10% CAGR

- **PNH (~30%)**
- **$9-10B Global Revenues**
- **>10B+ in pipeline peak sales potential**

2020 2023 2025 Beyond 2025

Illustrative, non risk-adjusted revenues, peak sales year varies by program

*2025 $9-10B target is at constant currencies (9/30/20 levels)*
Back-Up Slides
# Late Stage Pipeline Timelines

<table>
<thead>
<tr>
<th>Identifier</th>
<th>MOA</th>
<th>ROA</th>
<th>Indication</th>
<th>Phase</th>
<th>Study Start</th>
<th>Study End</th>
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<tr>
<td>SOLIRIS</td>
<td>Anti-C5</td>
<td>Q2W IV</td>
<td>Guillain Barre Syndrome</td>
<td>Ph3</td>
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<td>ULTOMIRIS (ravulizumab)</td>
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<td>Q1W SC</td>
<td>Paroxysmal Nocturnal Hemoglobinuria (PNH) Atypical Hemolytic Uremic Syndrome (aHUS)</td>
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<td>TLR 2Q '20 Filing 3Q '21</td>
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<td>Adults with COVID-19 who are hospitalized with severe pneumonia or ARDS</td>
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<td>Renal Basket Study</td>
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<td>ALXN1720</td>
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<td>AG10</td>
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<td>ALXN2040</td>
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<td>Ph2</td>
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<td>Paroxysmal Nocturnal Hemoglobinuria (PNH)</td>
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<td>TBD</td>
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<td>ANDEXXA</td>
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<td>Lymphoma (CTCL, PTCL, FL)</td>
<td>Ph2</td>
<td>PTLA Acquisition</td>
<td>TLR 1H '21</td>
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</table>

¹720 currently in HV Ph1 with topline readout estimated 1H '21 and subsequent DM and gMG trials to begin after that; ²1830 Ph1 HV program to reinitiate for SC formulation with WAIHA and gMG Ph2 programs to follow in 2021
## Near-Term Events Support All Three Pillars of Alexion’s Value Creation Strategy

### LEAD

<table>
<thead>
<tr>
<th>Event</th>
<th>Date</th>
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<tbody>
<tr>
<td>US IPR Settlement (Soliris Patents)</td>
<td>2Q 2020</td>
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<tr>
<td>ULTOMIRIS PNH Subcutaneous Ph3 Topline Data (PK)</td>
<td>2Q 2020</td>
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<td>ULTOMIRIS aHUS EMA Approval by EC</td>
<td>Mid 2020</td>
</tr>
<tr>
<td>ULTOMIRIS 100mg/ml Formulation FDA Approval</td>
<td>2H 2020</td>
</tr>
<tr>
<td>ULTOMIRIS Subcutaneous PNH/aHUS Launch</td>
<td>Mid 2022</td>
</tr>
</tbody>
</table>

### EXPAND

<table>
<thead>
<tr>
<th>Event</th>
<th>Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>ULTOMIRIS HSCT-TMA Ph3 Trial Initiation</td>
<td>4Q 2020</td>
</tr>
<tr>
<td>ULTOMIRIS Ph2 Renal Basket Trial Initiation</td>
<td>4Q 2020</td>
</tr>
<tr>
<td>ULTOMIRIS COVID-19 Ph3 Data</td>
<td>1H 2021</td>
</tr>
<tr>
<td>ULTOMIRIS gMG Ph3 Top Line Results</td>
<td>2H 2021</td>
</tr>
<tr>
<td>ULTOMIRIS ALS Ph3 Top Line Results</td>
<td>1H 2022</td>
</tr>
<tr>
<td>ULTOMIRIS NMOSD Ph3 Top Line Results</td>
<td>1H 2022</td>
</tr>
</tbody>
</table>

### DIVERSIFY

<table>
<thead>
<tr>
<th>Event</th>
<th>Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Portola Acquisition Close</td>
<td>3Q 2020</td>
</tr>
<tr>
<td>ALXN2040 C3G Ph2 Data</td>
<td>Mid 2020</td>
</tr>
<tr>
<td>AG10 Japan Ph3 Initiation</td>
<td>4Q 2020</td>
</tr>
<tr>
<td>CAEL-101 Ph3 Trial Initiation</td>
<td>2H 2020</td>
</tr>
<tr>
<td>ALXN1840 Wilson Ph3 Topline Data</td>
<td>1H 2021</td>
</tr>
<tr>
<td>ALXN2050 PNH Ph2 Topline Data</td>
<td>2H 2021</td>
</tr>
<tr>
<td>ALXN2040 GA Ph2 Initiation</td>
<td>2H 2021</td>
</tr>
<tr>
<td>AG10 Japan Ph3 Top Line Results</td>
<td>2H 2022</td>
</tr>
<tr>
<td>ALXN1840 Wilson Launch</td>
<td>2H 2022</td>
</tr>
</tbody>
</table>
## Growing and Advancing our Innovative Pipeline

### Significant Progress Made Developing A Robust, Value-Creating Pipeline

<table>
<thead>
<tr>
<th>Year</th>
<th>Assets</th>
<th>Clinical Programs</th>
<th>R&amp;D Expense</th>
<th>Program Related Spend:</th>
</tr>
</thead>
<tbody>
<tr>
<td>2017</td>
<td>2 Assets (2)</td>
<td>4 Clinical Programs</td>
<td>$736M Non-GAAP (1)</td>
<td>~50% C5 Expansion</td>
</tr>
<tr>
<td>2018 (3)</td>
<td>3 Assets</td>
<td>8 Clinical Programs</td>
<td>$646M Non-GAAP (1)</td>
<td>~50% Non-C5 Diversification</td>
</tr>
<tr>
<td>2019</td>
<td>8 Assets</td>
<td>14 Clinical Programs</td>
<td>$721M Non-GAAP (1)</td>
<td></td>
</tr>
<tr>
<td>2020</td>
<td>11 Assets</td>
<td>20+ Clinical Programs</td>
<td>$948M Non-GAAP (1)</td>
<td></td>
</tr>
</tbody>
</table>

(1) A reconciliation of GAAP to non-GAAP financial results is provided in the appendix and is available at www.alexion.com.

(2) Asset defined as a singular molecular entity;
(3) 2018 decrease in R&D spend related to pipeline strategy refocus;
(4) Mid point of 2020 guidance issued 7/30/20
Earlier Stage Pipeline Opportunities Beyond 2023 Launch

**LEAD & EXPAND in complement**
- **ALXN1720**
  - Ph2 Trial
  - To Initiate 4Q’20
  - Best in Class
  - C5 Inhibitor
- **DM**
- **Renal Basket**
  - gMG

**DIVERSIFY into new growth areas**
- **ALXN2040**
  - ~2.0M
  - U.S. & EU Diagnosed Prevalence
  - Ph2 Trial
  - To Initiate 4Q’20
  - Best in Class
  - Factor D
- **ALXN2050**
  - >200K
  - U.S. Diagnosed Prevalence
  - Ph2 Trial
  - To Initiate 1H’21
  - Best in Class
  - Factor Xa Reversal
- **ALXN1830**
  - ~100K
  - U.S. & EU Diagnosed Prevalence
  - Ph1 Trial
  - To Initiate 1H’21
  - Best in Class
  - FcRn

**Prevalence**
- ALXN1830 >200K
- ALXN2040 60-80K
- ALXN2050 ~50K
- ALXN2040 60-80K
- ALXN2050 >200K
- ALXN1830 ~100K
- ALXN2040 <200K
- ALXN2050 60-80K
- ALXN1830 >200K

**Additional**
- ALXN2040 ~2.0M
- U.S. & EU Diagnosed Prevalence
- ALXN2050 ~1.0M
- U.S. & EU Diagnosed Prevalence
- ALXN1830 >200K
Targeting Diseases of Complement Dysregulation

- Complement is a master sensor that discriminates between foreign or altered and healthy cell surfaces.
- Rationale for complement inhibition across multiple rare disease indications and therapeutic areas.
- Complexity of complement biology allows for multiple targeting approaches.
- Pursuing novel molecules and targets across terminal, lectin, and alternative pathways.

Denotes target ALXN is investigating
Capital Allocation / Business Development
Updated Approach to Capital Allocation

Significant Progress Since 2017 Allows an Updated and More Flexible Approach

- Alexion constantly evaluates our capital allocation approach
- R&D portfolio has grown substantially since 2017
  - 20+ total programs
  - Confidence in potential of 10 launches by 2023 enabling capital return
- Free cash flow generation has improved over the last three years, with high FCF conversion\(^1\)
- Allows Alexion to be more flexible on capital allocation strategy

Funding Expanding R&D Portfolio

Capital Allocation Priorities

**Share Repurchase** ↔ **Balance Sheet** (Reduce Net Debt) ↔ **Disciplined BD**

Committing to $500 – $550M of repurchases in 2020; increasing to at least 1/3 of FCF on average annually from 2021 - 2023

No near-term plan to pay a dividend given life-cycle

Near-term focus on successful Portola integration

Remain open to future BD that meet criteria for value creation and strategic fit

Robust Share Repurchase Program: Est. ~$3B & ~10% Reduction In Shares Outstanding Over Next Four Years\(^2\)

\(^1\)Free Cash Flow (FCF) defined as cash flow from operations less purchases of property, plant and equipment; FCF conversion defined as FCF divided by net income

\(^2\)Relative to 12/31/19 share count, excluding impact of new issuances
# Diversifying our Portfolio through Clinical and Commercial BD

<table>
<thead>
<tr>
<th>Asset</th>
<th>ALXN1840 (WTX-101) for treatment of Wilson Disease</th>
<th>ALXN2040 &amp; ALXN2050 Two oral Factor D inhibitors</th>
<th>ALXN1830 (SYNT-001) an anti-FcRn molecule</th>
<th>AG10 Rights to develop for ATTR in Japan only</th>
<th>CAEL-101 Co-develop for AL Amyloidosis with option to acquire</th>
<th>Elamipretide Co-develop for mitochondrial diseases</th>
<th>ABY-039 Co-develop anti-FcRn molecule</th>
</tr>
</thead>
<tbody>
<tr>
<td>Date</td>
<td>2Q 2018</td>
<td>4Q 2019</td>
<td>3Q 2018</td>
<td>3Q 2019</td>
<td>1Q 2019</td>
<td>4Q 2019</td>
<td>1Q 2019</td>
</tr>
<tr>
<td>Upfront $</td>
<td>~$1,200M</td>
<td>~$810M</td>
<td>~$700M</td>
<td>~$400M</td>
<td>~$50M</td>
<td>~$30M</td>
<td>~$30M</td>
</tr>
<tr>
<td>Alexion Ambition</td>
<td>Become standard of care for all Factor Xa inhibitor patients with a major or life-threatening bleeds</td>
<td>Redefine the standard of care with a transformative, highly specific copper binding agent</td>
<td>Deliver next generation &quot;oral&quot; complement inhibitor in a range of larger, rare indications</td>
<td>To be a leading SC FcRn in a variety of autoimmune indications</td>
<td>Bring a transformative small molecule to treat the root cause of ATTR to patients in Japan</td>
<td>Be the first targeted therapy to address underlying disease for AL amyloidosis patients</td>
<td></td>
</tr>
<tr>
<td>Progress to Date</td>
<td>ANDEXXA on market in US and EU Wave 1 Expanding geographically and potential label expansions</td>
<td>Ph3 trial fully enrolled Re-powered for superiority and added key secondary endpoints</td>
<td>2040: Ph2 C3G data; PNH with EVH Ph3 to initiate 2H 20</td>
<td>WAIHA Ph2 IV initiated; will transition to SC Successful SC data showing IgG reduction through Ph1 SC HV to support future gMG study</td>
<td>Expanding Ph3 study into Japan in 2020</td>
<td>Ph2 on-going with successful dose finding</td>
<td>Ph3 on track to initiate 2H 20</td>
</tr>
</tbody>
</table>

Above does not include deals made prior to 2018 (Taligen, Enobia, Xencor, Synageva, Halozyme) or pre-clinical deals/collaborations (Complement Pharma, Dicerna, Zealand, and Immune Pharma)
Pre-Clinical External Collaborations and Acquisitions Expand our Range of Expertise

<table>
<thead>
<tr>
<th>Collaborations</th>
<th>Acquisitions</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Dicerna™</strong></td>
<td><strong>Acquisition of ALXN2010 (anti-eotaxin-1) from Immune Pharma in 2019</strong></td>
</tr>
<tr>
<td><strong>• Collaboration to discover and develop SC delivered GalXC™ RNAi therapies for complement-mediated diseases</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Potential low volume injection with long half-life, supporting monthly+ dosing</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Initial agreement for two targets expanded to four in December 2019</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Partnership to co-discover a macrocyclic peptide inhibitor; platform already commercialized for other targets</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Peptide therapies offer a targeted approach with high potency and ease of administration</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Initial agreement for one target with option to expand to three additional</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Partnership to co-discover a therapeutic antibody against C6</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Expands our complement franchise with potential to address neurological disorders</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Circulating C6 in CNS leads to formation of MAC, which can cause neurodegeneration</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Eotaxin-1 is an eosinophil chemoattractant implicated in multiple inflammatory diseases</strong></td>
<td></td>
</tr>
<tr>
<td><strong>• Currently undergoing pre-clinical work to achieve subcutaneous administration</strong></td>
<td></td>
</tr>
</tbody>
</table>
ULTOMIRIS Modeling / Pricing Considerations
ULTOMIRIS Conversion Dynamic: Two Key Considerations

Conversion Loading Dose Dynamic

ULTOMIRIS vs. SOLIRIS U.S. Annual Cost Per Patient

- **PNH**
  - +10%
  - -10%

- **aHUS**
  - -20%
  - -33%

- Year 1: Loading dose + Maintenance Dosing
- Maintenance Dosing

Note: pricing discounts are approximations, not exact

- SOLIRIS indication-specific dosing: aHUS, gMG, NMOSD labeled dose higher than PNH
  - Drives indication-specific pricing differences when comparing SOLIRIS vs. ULTOMIRIS pricing
- ULTOMIRIS weight-based dosing

Quarter-on-quarter (QoQ) Variability

Infusion Timing Drives QoQ Variability

**Patient Sample 1**: Loading dose + 2 Maintenance Infusions

- **Patient Sample 2**: Loading dose + 1 Maintenance Infusion

- ULTOMIRIS every 8 week infusion schedule drives variability in quarterly patient treatment costs
- Expect quarterly variability to be negligible on year-over-year (YoY) revenue comparisons

Note: pricing discounts are approximations, not exact
The Alternative Complement Pathway & Renal Disease
The Alternative Pathway (AP) Is A Strong Mediator Of Abnormal Immune Response Across Several Renal Diseases

**Sampling of Potential Basket Trial Indications**

- **Lupus Nephritis (LN)**
  - ~90K US Prevalence
  - LN is caused by pathologic complement-fixing immune complex deposits and the production of autoantibodies; **Complement triggers acute inflammation in kidney**
  - Serum levels of complement biomarkers are linked with LN
  - AP inhibition plays role in mediating these aberrant immune responses

- **IgA Nephropathy**
  - ~60K US Prevalence
  - Alternative Pathway drives **inflammation and immune complex deposition** against under-glycosylated IgA in the kidney
  - AP inhibition can prevent activation of complement response to immune complexes, with potential to treat underlying pathophysiology & improve clinical outcomes

- **PMN**
  - ~60K US Prevalence
  - PMN is caused by pathologic auto antibodies against the PLA2R antigen on the podocyte surface of the kidney; over-amplifying the AP complement response
  - Complement targeted therapy has potential to treat the underlying pathophysiology and improve clinical outcomes

- **C3G**
  - <6K US Prevalence
  - C3G is caused by **uncontrolled and continued activation alternative pathway**, causing C3 deposition and inflammation in the kidney leading to kidney damage
  - Signals from ALXN2040 Ph2 C3G program support AP inhibition in C3G
Disease Descriptions
# Alexion Current Indications

<table>
<thead>
<tr>
<th>Indication</th>
<th>Description</th>
<th>Links</th>
</tr>
</thead>
<tbody>
<tr>
<td>PNH</td>
<td>Paroxysmal Nocturnal Hemoglobinuria&lt;br&gt;Chronic, debilitating, and potentially life-threatening ultra-rare blood disorder, with an average age of onset in the early 30s</td>
<td>more info</td>
</tr>
<tr>
<td>aHUS</td>
<td>atypical Hemolytic Uremic Syndrome&lt;br&gt;Ultra-rare, genetic, chronic, potentially life-threatening disease. Chronic uncontrolled complement activation results in thrombotic microangiopathy (TMA)</td>
<td>more info</td>
</tr>
<tr>
<td>gMG</td>
<td>Generalized Myasthenia Gravis&lt;br&gt;Debilitating, chronic, and progressive autoimmune neuromuscular disease.</td>
<td>more info</td>
</tr>
<tr>
<td>NMOSD</td>
<td>Neuromyelitis Optica Spectrum Disorder&lt;br&gt;Rare, devastating, complement-mediated disorder of the central nervous system characterized by relapses where each individual attack results in cumulative disability including blindness and paralysis, and sometimes premature death (primarily affects women)</td>
<td>more info</td>
</tr>
<tr>
<td>HPP</td>
<td>Hypophosphatemia&lt;br&gt;Inherited, progressive, ultra-rare metabolic disease in which patients experience devastating effects on multiple systems of the body, and face debilitating or life-threatening complications</td>
<td>more info</td>
</tr>
<tr>
<td>LAL-D</td>
<td>Liposomal Acid Lipase Deficiency&lt;br&gt;Genetic, chronic, and progressive ultra-rare metabolic disease in which infants, children, and adults experience continuous, uncontrolled accumulation of cholesteryl esters (CEs) and triglycerides (TGs) that may lead to multi-organ damage and premature death</td>
<td>more info</td>
</tr>
<tr>
<td>ANDEXXA</td>
<td>Coagulation factor Xa reversal (recombinant)&lt;br&gt;Reversal agent for life-threatening bleeds induced by factor Xa inhibitors</td>
<td>more info</td>
</tr>
</tbody>
</table>
## Alexion Pipeline Indications - I

<table>
<thead>
<tr>
<th>Indication</th>
<th>Description</th>
<th>Links</th>
</tr>
</thead>
<tbody>
<tr>
<td>WD</td>
<td>Wilson Disease</td>
<td>Rare, chronic, genetic, and potentially life-threatening liver disorder of impaired copper transport. The disorder is characterized by build-up of intra-cellular hepatic copper. Untreated, Wilson disease leads to various combinations and severity of hepatic, neurologic, and psychiatric symptoms, and can be fatal.</td>
</tr>
<tr>
<td>ALA</td>
<td>AL (Light-chain) Amyloidosis</td>
<td>A protein misfolding disorder in which B-cells produce incomplete ( \lambda ) and ( \kappa ) light chain antibodies which clump in certain organs / tissues (including heart, lungs, kidneys, nervous system, and liver, eventually causing organ damage and death.</td>
</tr>
<tr>
<td>PNH-E VH</td>
<td>Paroxysmal Nocturnal Hemoglobinuria with Extravascular Hemolysis</td>
<td>Chronic, debilitating, and potentially life-threatening ultra-rare blood disorder, with an average age of onset in the early 30s. EVH occurs when C3 opsonization of red blood cells causes macrophages to destroy those cells in tissue.</td>
</tr>
<tr>
<td>DM</td>
<td>Dermatomyositis</td>
<td>Progressive autoimmune condition that causes skin changes and muscle weakness. Symptoms can include a red skin rash around the eyelids, red bumps around the joints, and muscle weakness in the arms and legs. Dermatomyositis is most common in adults between ages 40 and 60, or in children between ages 5 and 15.</td>
</tr>
<tr>
<td>HSCT- TMA</td>
<td>Hematopoetic Stem Cell Transplant Thrombotic Micro-Angiopathy</td>
<td>A significant and often lethal complication of HSCT. The condition is a systemic, multifactorial disorder caused by endothelial cell damage induced by conditioning regimens, immunosuppressant therapies, infection, graft versus host disease (GVHD), and other factors associated with HSCT. HSCT-TMA prognosis is poor, with overall mortality reported as high as ~80-90%.</td>
</tr>
</tbody>
</table>
**Alexion Pipeline Indications - II**

<table>
<thead>
<tr>
<th>Indication</th>
<th>Description</th>
<th>Links</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>CM-TMA</strong> Complement-Mediated Thrombotic Micro-Angiopathy</td>
<td>Caused by abnormalities of regulation of the alternative pathway of complement activation. The indication describes a group of severe and chronic ultra-rare diseases that can cause progressive injury to vital organs—via damage to the walls of blood vessels and blood clots—potentially leading to organ failure and premature death. CM-TMA affects both adults and children and represents the population of patients with aHUS with or without triggers.</td>
<td></td>
</tr>
<tr>
<td><strong>COVID-19</strong> Severe Acute Respiratory Distress Syndrome in COVID-19 patients</td>
<td>Patients with severe illness include those who are hospitalized with severe pneumonia or acute respiratory distress syndrome. Evidence suggests that acute lung injury associated with COVID-19 may be mediated in part by complement pathway whereby elevated C5 ultimately leads to severe pneumonia, blood clots and multi-organ dysfunction in many advanced COVID patients.</td>
<td></td>
</tr>
<tr>
<td><strong>WAIHA</strong> Warm Auto-Immune Hemolytic Anemia</td>
<td>Rare autoimmune disorder caused by pathogenic Immunoglobulin G (IgG) antibodies that react with and cause the premature destruction of red blood cells at normal body temperature. The disease is often characterized by profound, and potentially life-threatening anemia and other acute complications.</td>
<td></td>
</tr>
<tr>
<td><strong>ATTR-CM</strong> Transthyretin Amyloidosis (ATTR) with Cardiomyopathy (ATTR-CM)</td>
<td>A progressive, fatal disease caused by the accumulation of misfolded tetrameric transthyretin (TTR) amyloid in the heart. Caused by the destabilization of TTR due to inherited mutations or aging, symptoms usually manifest later in life (age 50+), with median survival of three to five years from diagnosis.</td>
<td></td>
</tr>
</tbody>
</table>
### Alexion Pipeline Indications - III

<table>
<thead>
<tr>
<th>Indication</th>
<th>Description</th>
<th>Links</th>
</tr>
</thead>
<tbody>
<tr>
<td>LN</td>
<td>Lupus Nephritis</td>
<td>An inflammatory renal disease that is a severe complication of systemic lupus erythematosus (SLE), in which deposits of immune complexes (e.g., IgG and complement) accumulate in the kidney and lead to injury. Approximately 30% SLE patients develop LN, and up to 30% of patients are refractory to treatment and progress to end stage renal disease requiring dialysis/transplant within 15 years. There are no FDA approved therapies for LN.</td>
</tr>
<tr>
<td>PMN</td>
<td>Primary Membranous Nephropathy</td>
<td>Rare autoimmune disease characterized by autoantibodies to the podocyte membrane antigens PLA2R (~85%) and THSD7A (~5%) that causes nephrotic syndrome and chronic kidney disease. Approximately 30% of patients will progress to end stage renal disease within 10 years of diagnosis.</td>
</tr>
<tr>
<td>IgAN</td>
<td>IgA Nephropathy (IgAN)</td>
<td>A heterogenous disease in terms of clinical manifestations and progression and is the most common cause of primary glomerulonephritis. In IgAN, locally deposited immune complexes lead to activation of the complement cascade &amp; downstream endothelial organ damage. The Lectin and Alternative Pathways are believed to be the main driver of disease progression, which includes end stage renal disease and need for dialysis or transplant.</td>
</tr>
<tr>
<td>C3G</td>
<td>Complement 3 Glomerulopathy</td>
<td>Ultra-rare, heterogenous renal disease characterized by uncontrolled continued activation of fluid and/or solid phase alternative pathway causing C3 deposition and inflammation, leading to kidney damage.</td>
</tr>
<tr>
<td>ALS</td>
<td>Amyotrophic lateral sclerosis</td>
<td>A rare neurological disorder of progressive deterioration of nerve cells (motor neurons) in the brain and the spinal cord that control muscles throughout the body. Loss of motor neurons and muscle strength leads to loss of independence, paralysis and death, typically due to respiratory insufficiency.</td>
</tr>
</tbody>
</table>
Clinical Trials Appendix
**ALXN-1840 (WTX-101)**

<table>
<thead>
<tr>
<th>Trial</th>
<th>WTX101-301 trial (NCT03403205)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Population:</strong></td>
<td>Treatment-naïve and experienced Wilson disease patients</td>
</tr>
</tbody>
</table>
| **Arms:** | Active: ALXN1840 15-60mg once-daily (1-4 15mg tablets 1x / day)  
Control: standard of care (trientine, penicillamine, Zinc or combinations) |
| **Primary endpoint:** | % change in non-ceruloplasmin-bound copper from baseline to week 48 |
| **Size (n) / randomization:** | n=215, 1:1 randomization |
| **Treatment period:** | 48 weeks + LTE, up to 60 mos total |
| **Trial start date:** | 2/2018 |
| **Expected primary completion date:** | 2/2021 |
| **Clinicaltrials.gov link:** | WTX101-301 |
| **Trial stage:** | Phase 3 |

Directly sourced from www.clinicaltrials.gov
<table>
<thead>
<tr>
<th>Trial</th>
<th>CAEL101-301 trial (NCT04504825)</th>
<th>CAEL101-302 trial (NCT04512235)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Population:</td>
<td>1st line Mayo stage IIIb AL amyloidosis</td>
<td>1st line Mayo stage IIIa AL amyloidosis</td>
</tr>
<tr>
<td>Arms:</td>
<td>Active: CyBorD + CAEL-101 Control: CyBorD + Placebo</td>
<td>Active: CyBorD + CAEL-101 Control: CyBorD + Placebo</td>
</tr>
<tr>
<td>Primary endpoint:</td>
<td>Overall survival (OS)</td>
<td>Overall survival (OS)</td>
</tr>
<tr>
<td>Size (n) / randomization:</td>
<td>n=111, 2:1 randomization</td>
<td>n=267, 2:1 randomization</td>
</tr>
<tr>
<td>Treatment period:</td>
<td>Min 50 weeks</td>
<td>Min 50 weeks</td>
</tr>
<tr>
<td>Trial start date:</td>
<td>8/2020</td>
<td>8/2020</td>
</tr>
<tr>
<td>Expected primary completion date:</td>
<td>8/2022 (event-driven)</td>
<td>8/2022 (event-driven)</td>
</tr>
<tr>
<td>Clinicaltrials.gov link:</td>
<td>CAEL101-301</td>
<td>CAEL101-302</td>
</tr>
<tr>
<td>Trial stage:</td>
<td>Phase 3</td>
<td>Phase 3</td>
</tr>
</tbody>
</table>
## ULTOMIRIS ALS & HSCT-TMA

<table>
<thead>
<tr>
<th>Trial</th>
<th>ALXN1210-ALS-308 trial (NCT04248465)</th>
<th>ALXN1210-TMA-313 (NCT04543591)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Population:</strong></td>
<td>Adult participants with diagnosed ALS (sporadic or familial); ALS onset ≤ 36 months from Screening</td>
<td>Adult and adolescent participants with hematopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA)</td>
</tr>
</tbody>
</table>
| **Arms:** | Active: ULTOMIRIS  
Control: Placebo | Arm 1: ULTOMIRIS + Best Supportive Care  
Arm 2: Best Supportive Care |
| **Primary endpoint:** | Change From Baseline In Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R)  
Total Score | TMA Response |
| **Size (n) / randomization:** | n=354, 2:1 randomization | n=184, 1:1 randomized open label |
| **Treatment period:** | 50 weeks | 26 weeks |
| **Trial start date:** | 3/2020 | 9/2020 |
| **Expected primary completion date:** | 9/2022 | 8/2023 |
| **Clinicaltrials.gov link:** | ALXN1210-ALS-308 | ALXN1210-TMA-313 |
| **Trial stage:** | Phase 3 | Phase 3 |

Directly sourced from www.clinicaltrials.gov
## ULTOMIRIS gMG & NMOSD

<table>
<thead>
<tr>
<th>Trial</th>
<th>ALXN1210-MG-306 trial (NCT03920293)</th>
<th>ALXN1210-NMO-307 (NCT04170023)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Population:</strong></td>
<td>Participants with generalized myasthenia gravis; MG-ADL ≥ 6 at screening and randomization</td>
<td>Adult participants with Anti-aquaporin-4 antibody-positive and a diagnosis of NMOSD; at least 1 attack or relapse in last 12 months</td>
</tr>
<tr>
<td><strong>Arms:</strong></td>
<td>Active: ULTOMIRIS Control: Placebo</td>
<td>Single Arm</td>
</tr>
<tr>
<td><strong>Primary endpoint:</strong></td>
<td>Change From Baseline In Myasthenia Gravis-Activities Of Daily Living (MG-ADL) Total Score</td>
<td>Time To First Adjudicated On-Trial Relapse</td>
</tr>
<tr>
<td><strong>Size (n) / randomization:</strong></td>
<td>n=160, 1:1 randomized</td>
<td>n=55</td>
</tr>
<tr>
<td><strong>Treatment period:</strong></td>
<td>26 weeks</td>
<td>26 weeks to 2 years</td>
</tr>
<tr>
<td><strong>Trial start date:</strong></td>
<td>3/2019</td>
<td>12/2019</td>
</tr>
<tr>
<td><strong>Expected primary completion date:</strong></td>
<td>12/2021</td>
<td>11/2021</td>
</tr>
<tr>
<td><strong>Clinicaltrials.gov link:</strong></td>
<td>ALXN1210-MG-306</td>
<td>ALXN1210-NMO-307</td>
</tr>
<tr>
<td><strong>Trial stage:</strong></td>
<td>Phase 3</td>
<td>Phase 3</td>
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Directly sourced from www.clinicaltrials.gov
<table>
<thead>
<tr>
<th>Trial</th>
<th>ALXN1210-COV-305 (NCT04369469)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Population:</td>
<td>Adult patients with Coronavirus Disease 2019 (COVID-19) severe pneumonia, acute lung injury, or acute respiratory distress syndrome</td>
</tr>
</tbody>
</table>
| Arms: | Active: ULTOMIRIS plus best supportive care  
Control: Best supportive care |
| Primary endpoint: | Survival |
| Size (n) / randomization: | n=270, 2:1 randomized open label |
| Treatment period: | 29 days |
| Trial start date: | 5/2020 |
| Expected primary completion date: | 1H 2021 |
| Clinicaltrials.gov link: | ALXN1210-COV-305 |
| Trial stage: | Phase 3 |
## ALXN2040 (ACHN-4471) & ALXN2050 (ACHN5528)

<table>
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<tr>
<th>Trial</th>
<th>ALXN2040-PNH-301 trial (NCT04469465)</th>
<th>ACH228-110 (NCT04170023)</th>
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</thead>
<tbody>
<tr>
<td>Population:</td>
<td>Add-on therapy in paroxysmal nocturnal hemoglobinuria with clinically evident extravascular hemolysis</td>
<td>Treatment-experienced and treatment-naïve PNH patients, as monotherapy</td>
</tr>
</tbody>
</table>
| Arms: | Active: SOLIRIS or ULTOMIRIS + ALXN2040 (3x/day for ALXN2040)  
       | Control: SOLIRIS or ULTOMIRIS + Placebo (period I)  
       | and SOLIRIS or ULTOMIRIS + ALXN2040 (period II) | Single Arm |
| Primary endpoint: | Change in hemoglobin from baseline at week 12 | Change in hemoglobin from baseline at week 12 |
| Size (n) / randomization: | n=84, 2:1 randomization | n=26, open label |
| Treatment period: | 12 weeks (+12 wks for period II) + 1 yr LTE | 12 wks + LTE |
| Trial start date: | 12/2020 | 12/2019 |
| Expected primary completion date: | 10/2022 | 7/2021 |
| Clinicaltrials.gov link: | ALXN2040-PNH-301 | ACH228-110 |
| Trial stage: | Phase 3 | Phase 2 |

Directly sourced from www.clinicaltrials.gov
Board of Directors & Senior Leadership
Board Leadership overview

Board Skillset

- 10 of 10 directors have public company experience

- 10 of 10 directors have experience acquiring and/or divesting businesses and technologies and evaluating strategic corporate decisions

- 9 of 10 directors have C-suite level experience, with 6 current or former CEOs

- 8 of 10 directors have experience working for pharmaceutical or biopharmaceutical companies
  - One director is a highly respected and prominent biotechnology focused investor (Baker Bros.) and significant (2nd largest non-index) Alexion shareholder (4.05%)
  - One director is a former human resources executive who brings critical insight and experience to the design of executive compensation program

Board Committees

- Four standing Board committees - Audit and Finance, Leadership and Compensation, Nominating and Corporate Governance, and Science and Innovation

- S&I created to have full oversight over business development and internal/external R&D program goals and objectives by reviewing management’s progress and performance in achieving goals and objectives and mitigating associated risks; all members highly experienced in biotech, medicine and investing

Best Practices

- Strong emphasis on Board independence and strong Board and committee involvement, provides robust independent oversight of management, enterprise risk (including ESG risks and opportunities), and corporate operations

- Separated the roles of Chairman of the Board and CEO

Appointed 4 New Independent Directors with Deep Industry Experience since September 2017 including three industry veterans who are former and current CEOs

3 Biopharmaceutical Industry Veterans Added as New Directors Comprising of Former and Current CEOs

Strengthened And Refreshed Our Board of Directors to Align with Corporate Strategy
## Refresher Board of Directors

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<th>Title</th>
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<th>Experience/Qualifications</th>
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<td>Ludwig Hantson, Ph.D.</td>
<td>Chief Executive Officer</td>
<td>3 years</td>
<td>Accomplished healthcare executive with more than 30 years of experience in the biopharmaceutical industry, Director of Hologic, Ph.D. in motor rehabilitation and physical therapy, master's degree in physical education, and a certification in high secondary education, all from the University of Louvain in Belgium.</td>
</tr>
<tr>
<td>David Brennan</td>
<td>Independent Chairman of the Board</td>
<td>6 years</td>
<td>Extensive experience as an executive leader in the pharmaceutical industry, significant industry and regulatory knowledge from a more than 39-year career in the pharmaceutical industry and serving as a director on multiple public company and industry trade board. Former CEO of AstraZeneca, Director of Insmed, B.A. in business administration from Gettysburg College.</td>
</tr>
<tr>
<td>Andreas Rummelt, Ph.D.</td>
<td>Independent Director</td>
<td>10 years</td>
<td>More than 20 years in executive management positions in the pharmaceutical industry, broad understanding of international business operations particularly with respect to manufacturing, quality and technical matters, CEO of InterPharmaLink, Ph.D in pharmaceutical sciences from the University of Erlangen-Nuremberg (Germany).</td>
</tr>
<tr>
<td>Chris Coughlin</td>
<td>Independent Director</td>
<td>6 years</td>
<td>Extensive experience in complex financial and accounting matters, including public accounting and reporting, extensive operational experience managing, as well as evaluating and developing strategic goals for complex global organizations, Former EVP and CFO of Tyco, extensive public company board experience, named NACD Director of the Year in 2015. B.A. in Economics from St. John Fisher College and a Master's degree in secondary education, all from the University of Louvain in Belgium.</td>
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<td>Jack Mollen</td>
<td>Independent Director</td>
<td>6 years</td>
<td>Significant experience in executive compensation policy and administration including more than 30 years as chief human resources senior executive, Valued perspectives to the Board on matters of talent, executive compensation, benefits and leadership, Former EVP for Human Resources of EMC Corp., B.A. in Economics from St. John Fisher College and a Master's degree in Labor Relations from St. Francis College.</td>
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<td>Dr. Francois Nader, M.D.</td>
<td>Independent Director</td>
<td>3 years</td>
<td>Experienced biopharmaceutical CEO with strong background across both commercial and R&amp;D functions, Deep experience investing in biotechnology companies providing valuable insight in evaluating internal development product initiatives and external opportunities, Former CEO of NPS Pharma, Chairman of Prevail Therapeutics and Acceleron Pharma, Director of Moderna Therapeutics, Ph.D. in Immunology from Stanford University.</td>
</tr>
<tr>
<td>Dr. Paul Friedman, M.D.</td>
<td>Independent Director</td>
<td>3 years</td>
<td>Deep experience in research and clinical development, Extensive experience building and leading R&amp;D organizations, expanding company pipelines of assets, and overseeing the commercial development of innovative therapeutic products, Chairman and CEO of Madigal Pharmaceuticals and Director of Incyte, A.B. in Biology from Princeton University and M.D. from Harvard Medical School.</td>
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<tr>
<td>Dr. Deborah Dunsire, M.D.</td>
<td>Independent Director</td>
<td>3 years</td>
<td>More than 30 years of experience in the biopharmaceutical industry, including experience as a CEO if innovative companies focused on drug research and development, Extensive experience leading complex drug discovery, development and commercialization organizations, President and CEO of Lundbeck, Director of Ultragenyx, Medical degree from the University of Witwatersrand, Johannesburg, South Africa.</td>
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<tr>
<td>Felix Baker, Ph.D.</td>
<td>Independent Director</td>
<td>5 years</td>
<td>Broad experience serving as both a director and investor of biotechnology companies providing a strategic perspective of the industry, Significant industry and product development knowledge from a more than 25-year career investing in biotechnology, Co-Managing Member of Baker Bros. Advisors, Lead Independent Director of Kodiak Sciences and Kiniksa Pharmaceuticals, Director of Seattle Genetics, Ph.D and B.S. in Immunology from Stanford University.</td>
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<tr>
<td>Judy Reinsdorf</td>
<td>Independent Director</td>
<td>2 years</td>
<td>Strong corporate governance, compliance and legal expertise from leading legal functions at large U.S. public companies with global operations and in regulated industries, Broad experience in global compliance, strategic planning, data privacy, regulatory matters, as well as global M&amp;A experience, Former EVP and General Counsel of Johnson Controls, Bachelor's degree from the University of Rochester and J.D. from Cornell Law School.</td>
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5 of the 9 independent directors have been appointed in the last three years.
New Management Team Assembled Since 2017

Ludwig Hantson, Ph.D.
Chief Executive Officer
- Accomplished healthcare executive with more than 30 years of experience in the biopharmaceutical industry
- Ph.D. in motor rehabilitation and physical therapy, master's degree in physical education, and a certification in high secondary education, all from the University of Louvain in Belgium

Joined EC: March 2017

Aradhana Sarin, M.D.
Executive Vice President, Chief Financial Officer
- Responsible for overseeing global financial management, treasury, internal audit, corporate strategy, business development, investor relations, security activities, and business operations, including corporate planning
- Completed medical training at the University of Delhi and received her MBA from Stanford Business School

Joined EC: February 2019

Tanisha Carino, Ph.D.
Executive Vice President, Chief Corporate Affairs Officer
- Responsible for global government relations, policy and communications
- Ph.D. in health policy from Johns Hopkins University, and is associate faculty at the Johns Hopkins Bloomberg School of Public Health

Joined EC: November 2019

Indrani Franchini, J.D.
Executive Vice President, Chief Compliance Officer
- Responsible for leading Alexion’s global compliance program and co-leads the Global Corporate Compliance Committee
- J.D. from the University of Michigan Law School and a Bachelor of Arts from Princeton University, as well as a Fulbright Fellow at Kyushu University

Joined EC: June 2017

Rana Strellis
Senior Vice President, Global Culture and Corporate Social Responsibility
- Rana Strellis is Senior Vice President, Global Culture and Corporate Social Responsibility of Alexion.
- MBA from Cornell University and BA in Economics from the University of Illinois at Urbana-Champaign

Joined EC: October 2017

John Orloff, M.D.
Executive Vice President, Head of Research & Development
- Responsible for global R&D, Regulatory, and Medical Affairs at Alexion, including enhancing R&D productivity and supporting business development
- Bachelor of Arts from Dartmouth College, and a M.D. from the University of Vermont College of Medicine. Completed medical training at the University of Pittsburgh Medical Center and Yale University School of Medicine

Joined EC: June 2017

Ellen Chiniara, J.D.
Executive Vice President, Chief Legal Officer and Corporate Secretary
- Responsible for overseeing all global legal matters for the Company
- J.D. from Stanford University’s School of Law and Bachelor’s Degree from Bryn Mawr College, as well as a graduate fellow at Yale University in Slavic Languages

Joined EC: February 2018

Morgan Sanford
Chief of Staff to the Chief Executive Officer
- Organizes and prioritizes critical issues and required information for the CEO and Executive Committee
- MBA from the Leonard N. Stern School at New York University and her Bachelor’s degree in Neuroscience from Hamilton College

Joined EC: March 2020

7 of 11 Executive team members are women; 53% of total workforce are female
Sustainability At Alexion
Alexion’s COVID-19 Response

Our Ongoing Commitment

To the Broader Community:
- Alexion is committed to assisting communities and medical systems in their efforts to address the public health crisis caused by the COVID-19 pandemic and is taking a holistic approach to supporting our global communities across a range of areas of need.

To Our Patients:
- We remain committed to our mission of serving patients with rare disease
- We remain focused on continuous supply of our medicines to our patients who rely on them
- We are steadfast in ensuring the integrity of our ongoing clinical trials and mitigating risks of interruption

To Our Employees:
- We continue to be dedicated to the health and safety of our global employees

Alexion’s Response

Initiated Phase 3 Randomized Controlled Trial:
- Phase 3 trial investigating the use of ULTOMIRIS in patients with severe COVID-19
- Early pre-clinical and anecdotal evidence from independent investigator-sponsored work suggests a role for complement in treating severe COVID-19

Providing Support:
- Opened emergency Expanded Access programs for some patients’ urgent needs
- Leveraged robust supply chain to ensure sufficient inventory for critical life-saving medications
- Remain committed to support community and local healthcare efforts to address COVID-19 public health crisis, including donation of lab equipment and instruments to hospital labs
- Alexion Charitable Foundation donated $500,000 across three nonprofit organizations to global COVID-19 response funds
- All non-essential employees (not in labs or manufacturing) have worked from home to ensure their safety; established policies / procedures to support more workday flexibility
- Programs put in place to foster employees’ connectedness and provide support needed.
Corporate Social Responsibility (CSR) at Alexion

SUPPORTING OUR MISSION TO TRANSFORM THE LIVES OF PEOPLE AFFlicted BY RARE AND DEVASTATING DISEASE WHILE CREATING VALUE FOR ALL OUR STAKEHOLDERS.

SERVE COMMUNITIES AND SUSTAIN OUR PLANET
We invest in our communities and shared planet in support of those who depend on us today and for generations that follow.

TRANSFORM PATIENT LIVES
We urgently seek to understand patient journeys, find answers, and collaborate to deliver access to therapies that change lives.

ADVANCE OUR PEOPLE AND OUR COMPANY
We aspire to become the most rewarding company to work for, embracing belonging, and governing and managing our business to return value to our stakeholders.

REDEFINE WHAT IT MEANS TO LIVE WITH A RARE DISEASE
We pioneered complement biology, spurring new treatments for devastating disorders. We work to advance healthcare through innovative diagnostics and proactive transparency.

ETHICS & COMPLIANCE: OUR FOUNDATION
We build trust when we make the right choices and act with integrity. Our unwavering commitment to ethics, quality and compliance improves our ability to serve patients and enhances our reputation and competitive advantage.

CSR IS AN ACRONYM FOR CORPORATE SOCIAL RESPONSIBILITY

Recognition

Corporate ESG Performance

Prime

Awarded to companies with an ESG performance above the sector-specific Prime threshold, which means that they fulfill ambitious absolute performance requirements.

RATED BY ISS ESG

SUSTAINALYTICS

<table>
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<th>Rank</th>
<th>Percentile</th>
</tr>
</thead>
<tbody>
<tr>
<td>Global Universe</td>
<td>4208 out of 12711</td>
</tr>
<tr>
<td>Pharmaceuticals (Industry Group)</td>
<td>47 out of 751</td>
</tr>
<tr>
<td>Biotechnology (Subindustry)</td>
<td>5 out of 352</td>
</tr>
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## Corporate Social Responsibility (CSR) at Alexion

In 2019, Alexion formalized aspirations and accompanying metrics aligned with our CSR-STAR Platform. Even though these formal aspirations and metrics were recently established, our teams have been committed to many of these areas of focus since the company’s founding. For most areas, 2019 will serve as the baseline year against which we will measure progress, with the exception of two environmental metrics for which the baseline year will be 2020. Moving forward, we plan to report yearly progress against these aspirations in our annual CSR report.

### Table of Focus Areas, Aspirations, Metrics, and Statuses

<table>
<thead>
<tr>
<th>FOCUS AREA</th>
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<th>METRIC</th>
<th>2019 STATUS</th>
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CSR Reporting Principles

- Utilize **best practice frameworks** to anchor content selection.
- Focus on **Shared Value** – the premise that companies can profit by doing “good,” supported by examples of **Alexion’s core business** activities.
- Proactively share information sought by **internal and external stakeholders**.
- Develop a tool that may be used by our colleagues to **tell our Alexion CSR story**.
- Create a narrative that instills employees with **pride** and external stakeholders with **trust**.

**Sustainability Accounting Standards Board (SASB)**

- Gaining traction, considered advanced
- Tailored to investors and lenders
- Highly sector specific

**Global Reporting Initiative (GRI) Standards**

- Most widely adopted CSR reporting framework in the world
- Used by 75% of the world’s largest companies
- Caters to information needs of a broad cross section of stakeholders
Our CSR Evolution

Q3 2017 – 2019

- Created and launched Alexion's CSR-STAR Platform and long-term, sustainable strategy
- Translated CSR-STAR into 8 languages
- Created CSR-Steering Committee & Grew CSR Team
- Participated in internal & external CSR education and awareness events
- Engaged for first time with external CSR Rating/Rankings agencies
- Established membership with key external CSR industry groups

First Half | 2020

- Launched Volunteer Tracking Platform & Volunteer Paid Time Off
- Launched Alexion Charitable Foundation (ACF)
- Deployed COVID-19 philanthropic relief (Corporate Giving & ACF)
- Launched first CSR Report
- Launched COVID-19 virtual volunteering program
- Kicked-off Alexion’s Global Brain Health strategy development
- Kicked-off creation of global CSR overview video for use with internal and external stakeholders

Second Half | 2020

- Completing 2020 CSR Benchmarking Assessment
- Identifying CSR/ESG Reporting Gaps and Alexion-specific areas of focus
- Leading Alexion’s first Virtual Global Week of Service
- Developing ACF Thought Leadership Platform around mental health within the Rare Disease Community
- Develop ACF Global Disaster Relief Policy (Planned)
- Expand Alexion’s Matching Gifts program (Planned)
At Alexion, Diversity is having a seat at the table. Inclusion is having a voice. Belonging is having that voice be heard. Ensuring all of our colleagues feel a sense of belonging will enable us to magnetize and incubate diverse talent which will allow us to harness diverse insights that fuel innovation and create value for the patients we serve.

“Establishing the Chief Diversity Officer role at Alexion is an important next step in our continued efforts to cultivate diversity, inclusion and a unique sense of belonging at the company, all of which enhances our ability to deliver on our mission of transforming the lives of patients with rare diseases and devastating conditions.”

-Ludwig Hantson, Ph.D., CEO, Alexion
### ALEXION PHARMACEUTICALS, INC.

#### TABLE 1: CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(in millions, except per share amounts)

(unaudited)

<table>
<thead>
<tr>
<th></th>
<th>Three months ended</th>
<th></th>
<th>Six months ended</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>June 30, 2020</td>
<td>2019</td>
<td>June 30, 2020</td>
<td>2019</td>
</tr>
<tr>
<td>Net product sales</td>
<td>$1,444.5</td>
<td>$1,202.5</td>
<td>$2,889.1</td>
<td>$2,342.7</td>
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<td>0.1</td>
<td>0.8</td>
<td>0.3</td>
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<tr>
<td>Total revenues</td>
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<td>2,889.4</td>
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<tr>
<td>Costs and expenses:</td>
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</tr>
<tr>
<td>Cost of sales (exclusive of amortization of purchased intangible assets)</td>
<td>144.9</td>
<td>99.2</td>
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<tr>
<td>Research and development</td>
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<td>187.6</td>
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<td>580.8</td>
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<tr>
<td>Acquired in-process research and development</td>
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<td>(4.1)</td>
<td>-</td>
<td>(4.1)</td>
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<tr>
<td>Amortization of purchased intangible assets</td>
<td>73.7</td>
<td>80.1</td>
<td>147.4</td>
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<tr>
<td>Change in fair value of contingent consideration</td>
<td>15.8</td>
<td>6.1</td>
<td>21.6</td>
<td>(22.6)</td>
</tr>
<tr>
<td>Acquisition-related costs</td>
<td>4.6</td>
<td>-</td>
<td>42.7</td>
<td>-</td>
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<tr>
<td>Restructuring expenses</td>
<td>-</td>
<td>2.5</td>
<td>(0.8)</td>
<td>11.6</td>
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<tr>
<td>Impairment of intangible assets</td>
<td>2,053.3</td>
<td>-</td>
<td>2,053.3</td>
<td>-</td>
</tr>
<tr>
<td>Total costs and expenses</td>
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<td>670.7</td>
<td>3,564.1</td>
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<td>Operating (loss) income</td>
<td>(1,370.2)</td>
<td>532.6</td>
<td>(674.7)</td>
<td>1,049.4</td>
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<tr>
<td>Other income and expense:</td>
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<td></td>
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<tr>
<td>Investment income (expense)</td>
<td>41.5</td>
<td>(14.9)</td>
<td>36.3</td>
<td>27.6</td>
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<tr>
<td>Interest expense</td>
<td>(23.6)</td>
<td>(18.3)</td>
<td>(49.4)</td>
<td>(38.2)</td>
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<td>0.1</td>
<td>(0.7)</td>
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<td>(Loss) income before income taxes</td>
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<td>499.5</td>
<td>(688.5)</td>
<td>1,041.3</td>
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<td>39.7</td>
<td>(178.0)</td>
<td>(6.4)</td>
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<td>($1,636.1)</td>
<td>$459.8</td>
<td>($567.5)</td>
<td>$1,047.7</td>
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<td>Earnings (loss) per common share</td>
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<tr>
<td>Basic</td>
<td>($4.84)</td>
<td>$2.05</td>
<td>($2.31)</td>
<td>$4.68</td>
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<tr>
<td>Diluted</td>
<td>($4.84)</td>
<td>$2.04</td>
<td>($2.31)</td>
<td>$4.64</td>
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<tr>
<td>Shares used in computing earnings (loss) per common share</td>
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<td></td>
<td></td>
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<tr>
<td>Basic</td>
<td>220.6</td>
<td>224.2</td>
<td>221.1</td>
<td>224.0</td>
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<tr>
<td>Diluted</td>
<td>220.6</td>
<td>225.8</td>
<td>221.1</td>
<td>225.7</td>
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<tr>
<td></td>
<td>Three months ended</td>
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<tr>
<td>--------------------------</td>
<td>--------------------</td>
<td>------------------</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>June 30, 2020</td>
<td>June 30, 2019</td>
<td></td>
<td></td>
</tr>
<tr>
<td>GAAP net (loss) income</td>
<td>$1,068.1</td>
<td>459.8</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Before tax adjustments:</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Cost of sales:</td>
<td></td>
<td></td>
<td></td>
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</tr>
<tr>
<td>Share-based compensation</td>
<td>3.1</td>
<td>3.5</td>
<td></td>
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</tr>
<tr>
<td>Research and development expense:</td>
<td></td>
<td></td>
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<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>16.5</td>
<td>13.9</td>
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<tr>
<td>Upfront payments related to licenses and other strategic agreements</td>
<td>—</td>
<td>25.0</td>
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<td></td>
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<tr>
<td>Selling, general and administrative expense:</td>
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<td></td>
<td></td>
<td></td>
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<tr>
<td>Share-based compensation</td>
<td>47.8</td>
<td>43.5</td>
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<tr>
<td>Litigation charges</td>
<td>—</td>
<td>—</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Amortization of purchased intangible assets</td>
<td>73.7</td>
<td>80.1</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Change in fair value of contingent consideration</td>
<td>15.8</td>
<td>6.1</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Acquisition-related costs</td>
<td>4.6</td>
<td>—</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Restructuring expenses</td>
<td>—</td>
<td>2.5</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Impairment of intangible assets</td>
<td>2,053.3</td>
<td>2,053.3</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Investment income (expense):</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>(Gains) and losses related to strategic equity investments</td>
<td>(35.0)</td>
<td>25.2</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Other income and (expense):</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Adjustments to income tax expense</td>
<td>(409.5)</td>
<td>(50.5)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Non-GAAP net income</td>
<td>$702.2</td>
<td>$605.0</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th></th>
<th>2020</th>
<th>2019</th>
</tr>
</thead>
<tbody>
<tr>
<td>GAAP earnings (loss) per common share - diluted</td>
<td>$ (4.84)</td>
<td>$ 2.04</td>
</tr>
<tr>
<td>Non-GAAP earnings per common share - diluted</td>
<td>$ 3.11</td>
<td>$ 2.64</td>
</tr>
</tbody>
</table>

| Shares used in computing diluted earnings (loss) per common share (GAAP) | 220.6 | 225.6 |
| Shares used in computing diluted earnings per common share (non-GAAP) | 225.7 | 228.9 |
(1) During the three months ended June 30, 2019, we recorded expense of $25.0 million in connection with an upfront payment on a strategic agreement that we entered into with Affibody AB (Affibody). During the six months ended June 30, 2019, we recorded expense of $46.2 million in connection with upfront payments on strategic agreements that we entered into with Affibody and Zealand Pharma A/S.

(2) During the six months ended June 30, 2020, we recorded $21.5 million in litigation charges in connection with legal proceedings.

(3) Changes in the fair value of contingent consideration expense for the three and six months ended June 30, 2020 as well as the six months ended June 30, 2019 include the impact of changes in the expected timing of achieving contingent milestones, in addition to the interest component related to the passage of time. For the three months ended June 30, 2019, changes in fair value of contingent consideration expense reflected only the interest component of contingent consideration related to the passage of time.

(4) For the three and six months ended June 30, 2020, we recorded $4.6 million and $42.7 million, respectively, of acquisition-related costs in connection with the Achillion Pharmaceuticals, Inc. and Portola Pharmaceuticals, Inc. acquisitions. Acquisition-related costs primarily consist of Achillion and Portola transaction costs, costs associated with the accelerated vesting of stock options previously granted to Achillion employees and Achillion restructuring-related costs.

(5) In the second quarter 2020, we recognized impairment charges of $2,053.3 million, primarily related to our KANUMA intangible asset.

(6) Alexion’s non-GAAP income tax expense for the three and six months ended June 30, 2020 and 2019 excludes the tax effect of pre-tax adjustments to GAAP profit. Non-GAAP income tax expense for the six months ended June 30, 2019 also excludes certain one-time tax benefits of $95.7 million and $30.3 million associated with a tax election made with respect to intellectual property of Wilson and a release of an existing valuation allowance, respectively.
**ALEXION PHARMACEUTICALS, INC.**

**TABLE 3: RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL GUIDANCE**

(In millions, except per share amounts and percentages)

<table>
<thead>
<tr>
<th></th>
<th>Twelve months ending</th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>December 31, 2020</td>
<td>Low</td>
<td>High</td>
</tr>
<tr>
<td>GAAP net income</td>
<td>$214</td>
<td>$200</td>
<td></td>
</tr>
<tr>
<td><strong>Before tax adjustments:</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>295</td>
<td>282</td>
<td></td>
</tr>
<tr>
<td>Impairment of intangible assets</td>
<td>2,053</td>
<td>2,053</td>
<td></td>
</tr>
<tr>
<td>Amortization of purchased intangible assets</td>
<td>202</td>
<td>202</td>
<td></td>
</tr>
<tr>
<td>Acquisition-related costs</td>
<td>131</td>
<td>131</td>
<td></td>
</tr>
<tr>
<td>Change in fair value of contingent consideration</td>
<td>31</td>
<td>31</td>
<td></td>
</tr>
<tr>
<td>Restructuring expenses</td>
<td>(1)</td>
<td>(1)</td>
<td></td>
</tr>
<tr>
<td>(Gains) and losses related to strategic equity investments</td>
<td>(26)</td>
<td>(26)</td>
<td></td>
</tr>
<tr>
<td>Litigation charges</td>
<td>22</td>
<td>22</td>
<td></td>
</tr>
<tr>
<td>Adjustments to income tax expense</td>
<td>(515)</td>
<td>(515)</td>
<td></td>
</tr>
<tr>
<td><strong>Non-GAAP net income</strong></td>
<td><strong>$2,402</strong></td>
<td><strong>$2,400</strong></td>
<td></td>
</tr>
<tr>
<td>Diluted GAAP earnings per common share</td>
<td>$0.96</td>
<td>$1.30</td>
<td></td>
</tr>
<tr>
<td>Diluted non-GAAP earnings per common share</td>
<td>$10.65</td>
<td>$10.90</td>
<td></td>
</tr>
<tr>
<td><strong>Costs and expenses and margin (% total revenues):</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>GAAP research and development expense</td>
<td>19.2 %</td>
<td>18.1 %</td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>1.7 %</td>
<td>1.6 %</td>
<td></td>
</tr>
<tr>
<td>Restructuring related expenses</td>
<td>0.0 %</td>
<td>0.0 %</td>
<td></td>
</tr>
<tr>
<td><strong>Non-GAAP research and development expense</strong></td>
<td><strong>17.5%</strong></td>
<td><strong>16.5%</strong></td>
<td></td>
</tr>
<tr>
<td>GAAP selling, general and administrative expense</td>
<td>25.7 %</td>
<td>24.5 %</td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>3.3 %</td>
<td>3.1 %</td>
<td></td>
</tr>
<tr>
<td>Restructuring related expenses</td>
<td>0.0 %</td>
<td>0.0 %</td>
<td></td>
</tr>
<tr>
<td>Litigation charges</td>
<td>0.4 %</td>
<td>0.4 %</td>
<td></td>
</tr>
<tr>
<td><strong>Non-GAAP selling, general and administrative expense</strong></td>
<td><strong>22.0 %</strong></td>
<td><strong>21.0 %</strong></td>
<td></td>
</tr>
<tr>
<td>GAAP operating margin</td>
<td>3.8 %</td>
<td>5.4 %</td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>5.3 %</td>
<td>5.0 %</td>
<td></td>
</tr>
<tr>
<td>Litigation charges</td>
<td>0.4 %</td>
<td>0.4 %</td>
<td></td>
</tr>
<tr>
<td>Impairment of intangible assets</td>
<td>37.0 %</td>
<td>36.7 %</td>
<td></td>
</tr>
<tr>
<td>Amortization of purchased intangible assets</td>
<td>3.6 %</td>
<td>3.6 %</td>
<td></td>
</tr>
<tr>
<td>Acquisition-related costs</td>
<td>2.4 %</td>
<td>2.3 %</td>
<td></td>
</tr>
<tr>
<td>Change in fair value of contingent consideration</td>
<td>0.6 %</td>
<td>0.6 %</td>
<td></td>
</tr>
<tr>
<td>Restructuring expenses</td>
<td>0.0 %</td>
<td>0.0 %</td>
<td></td>
</tr>
<tr>
<td><strong>Non-GAAP operating margin</strong></td>
<td><strong>53.0%</strong></td>
<td><strong>54.0%</strong></td>
<td></td>
</tr>
<tr>
<td>Income tax expense (% of income before income taxes)</td>
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</tr>
<tr>
<td>GAAP income tax expense (benefit)</td>
<td>(26.0%)</td>
<td>(27.0%)</td>
<td></td>
</tr>
<tr>
<td>Tax effect of pre-tax adjustments to GAAP net income</td>
<td>4.5%</td>
<td>4.5%</td>
<td></td>
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<tr>
<td><strong>Non-GAAP income tax expense</strong></td>
<td><strong>18.5%</strong></td>
<td><strong>15.5%</strong></td>
<td></td>
</tr>
</tbody>
</table>

*Amounts may not total due to rounding.*
<table>
<thead>
<tr>
<th></th>
<th>Three months ended</th>
<th></th>
<th>Six months ended</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>June 30,</td>
<td></td>
<td>June 30,</td>
<td></td>
</tr>
<tr>
<td></td>
<td>2020</td>
<td>2019</td>
<td>2020</td>
<td>2019</td>
</tr>
<tr>
<td>SOURIS</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>United States</td>
<td>$ 553.3</td>
<td>$ 496.3</td>
<td>$ 1,109.6</td>
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<td>Europe</td>
<td>247.9</td>
<td>290.2</td>
<td>511.4</td>
<td>544.7</td>
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<tr>
<td>Asia Pacific</td>
<td>82.4</td>
<td>110.3</td>
<td>169.5</td>
<td>211.2</td>
</tr>
<tr>
<td>Rest of World</td>
<td>91.9</td>
<td>94.0</td>
<td>208.0</td>
<td>226.9</td>
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<tr>
<td>Total SOURIS</td>
<td>$ 975.5</td>
<td>$ 980.8</td>
<td>$ 1,958.4</td>
<td>$ 1,942.8</td>
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<tr>
<td>ULTOMIRIS</td>
<td></td>
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<td></td>
</tr>
<tr>
<td>United States</td>
<td>$ 158.1</td>
<td>$ 54.2</td>
<td>$ 289.6</td>
<td>$ 78.8</td>
</tr>
<tr>
<td>Europe</td>
<td>32.0</td>
<td>—</td>
<td>65.8</td>
<td>—</td>
</tr>
<tr>
<td>Asia Pacific</td>
<td>59.6</td>
<td>—</td>
<td>116.7</td>
<td>—</td>
</tr>
<tr>
<td>Rest of World</td>
<td>1.4</td>
<td>—</td>
<td>1.8</td>
<td>—</td>
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<tr>
<td>Total ULTOMIRIS</td>
<td>$ 251.1</td>
<td>$ 54.2</td>
<td>$ 473.9</td>
<td>$ 78.8</td>
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<tr>
<td>STRENSIQ</td>
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<td></td>
</tr>
<tr>
<td>United States</td>
<td>$ 140.7</td>
<td>$ 106.2</td>
<td>$ 268.8</td>
<td>$ 205.7</td>
</tr>
<tr>
<td>Europe</td>
<td>18.3</td>
<td>19.5</td>
<td>42.3</td>
<td>37.0</td>
</tr>
<tr>
<td>Asia Pacific</td>
<td>15.0</td>
<td>12.1</td>
<td>28.6</td>
<td>22.0</td>
</tr>
<tr>
<td>Rest of World</td>
<td>10.3</td>
<td>3.5</td>
<td>16.8</td>
<td>6.7</td>
</tr>
<tr>
<td>Total STRENSIQ</td>
<td>$ 184.3</td>
<td>$ 141.3</td>
<td>$ 396.5</td>
<td>$ 271.4</td>
</tr>
<tr>
<td>KANUMA</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>United States</td>
<td>$ 15.4</td>
<td>$ 15.3</td>
<td>$ 31.8</td>
<td>$ 20.1</td>
</tr>
<tr>
<td>Europe</td>
<td>8.4</td>
<td>6.8</td>
<td>15.0</td>
<td>13.1</td>
</tr>
<tr>
<td>Asia Pacific</td>
<td>0.9</td>
<td>1.3</td>
<td>1.8</td>
<td>2.1</td>
</tr>
<tr>
<td>Rest of World</td>
<td>8.9</td>
<td>2.8</td>
<td>10.6</td>
<td>5.4</td>
</tr>
<tr>
<td>Total KANUMA</td>
<td>$ 33.6</td>
<td>$ 20.2</td>
<td>$ 60.3</td>
<td>$ 49.7</td>
</tr>
<tr>
<td><strong>Net Product Sales</strong></td>
<td>$ 867.5</td>
<td>$ 672.0</td>
<td>$ 1,699.7</td>
<td>$ 1,273.6</td>
</tr>
<tr>
<td>United States</td>
<td>366.6</td>
<td>306.5</td>
<td>635.4</td>
<td>594.8</td>
</tr>
<tr>
<td>Europe</td>
<td>306.5</td>
<td>306.5</td>
<td>635.4</td>
<td>594.8</td>
</tr>
<tr>
<td>Asia Pacific</td>
<td>157.9</td>
<td>123.7</td>
<td>316.6</td>
<td>235.3</td>
</tr>
<tr>
<td>Rest of World</td>
<td>112.5</td>
<td>103.3</td>
<td>237.4</td>
<td>239.0</td>
</tr>
<tr>
<td><strong>Total Net Product Sales</strong></td>
<td>$ 1,415.5</td>
<td>$ 1,202.5</td>
<td>$ 2,889.1</td>
<td>$ 2,342.7</td>
</tr>
</tbody>
</table>
### ALEXION PHARMACEUTICALS, INC.
### TABLE 5: CONDENSED CONSOLIDATED BALANCE SHEETS
### (in millions)
### (unaudited)

<table>
<thead>
<tr>
<th></th>
<th>June 30, 2020</th>
<th>December 31, 2019</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash and cash equivalents</td>
<td>$2,825.0</td>
<td>$2,685.6</td>
</tr>
<tr>
<td>Marketable securities</td>
<td>26.8</td>
<td>64.0</td>
</tr>
<tr>
<td>Trade accounts receivable, net</td>
<td>1,372.2</td>
<td>1,243.2</td>
</tr>
<tr>
<td>Inventories</td>
<td>577.7</td>
<td>627.6</td>
</tr>
<tr>
<td>Prepaid expenses and other current assets</td>
<td>886.2</td>
<td>456.1</td>
</tr>
<tr>
<td>Property, plant and equipment, net</td>
<td>1,186.4</td>
<td>1,163.3</td>
</tr>
<tr>
<td>Intangible assets, net</td>
<td>2,059.7</td>
<td>3,344.3</td>
</tr>
<tr>
<td>Goodwill</td>
<td>5,075.2</td>
<td>5,037.4</td>
</tr>
<tr>
<td>Right of use operating assets</td>
<td>209.9</td>
<td>204.0</td>
</tr>
<tr>
<td>Deferred tax assets</td>
<td>2,332.4</td>
<td>2,290.2</td>
</tr>
<tr>
<td>Other assets</td>
<td>461.7</td>
<td>429.0</td>
</tr>
<tr>
<td><strong>Total assets</strong></td>
<td>$16,703.2</td>
<td>$17,544.6</td>
</tr>
<tr>
<td>Accounts payable and accrued expenses</td>
<td>$861.6</td>
<td>$966.7</td>
</tr>
<tr>
<td>Current portion of long-term debt</td>
<td>126.8</td>
<td>126.7</td>
</tr>
<tr>
<td>Other current liabilities</td>
<td>131.7</td>
<td>100.9</td>
</tr>
<tr>
<td>Long-term debt, less current portion</td>
<td>2,311.6</td>
<td>2,375.0</td>
</tr>
<tr>
<td>Contingent consideration</td>
<td>374.7</td>
<td>192.4</td>
</tr>
<tr>
<td>Deferred tax liabilities</td>
<td>1,946.8</td>
<td>2,081.4</td>
</tr>
<tr>
<td>Noncurrent operating lease liabilities</td>
<td>169.4</td>
<td>164.1</td>
</tr>
<tr>
<td>Other liabilities</td>
<td>289.8</td>
<td>265.6</td>
</tr>
<tr>
<td><strong>Total liabilities</strong></td>
<td>6,212.4</td>
<td>6,272.8</td>
</tr>
<tr>
<td><strong>Total stockholders’ equity</strong></td>
<td>10,400.8</td>
<td>11,271.8</td>
</tr>
<tr>
<td><strong>Total liabilities and stockholders’ equity</strong></td>
<td>$16,703.2</td>
<td>$17,544.6</td>
</tr>
</tbody>
</table>
### APPENDIX

#### TABLE 6: CONDENSED CONSOLIDATED STATEMENT OF CASH FLOWS

(in millions/unaudited)

<table>
<thead>
<tr>
<th></th>
<th>2020</th>
<th>2019</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cash flows from operating activities</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Net (loss) income</td>
<td>$(510.5)</td>
<td>$1,047.7</td>
</tr>
<tr>
<td>Adjustments to reconcile net (loss) income to net cash flows from operating activities:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Depreciation and amortization</td>
<td>179.1</td>
<td>193.7</td>
</tr>
<tr>
<td>Change in fair value of contingent consideration</td>
<td>21.6</td>
<td>(22.6)</td>
</tr>
<tr>
<td>Share-based compensation expense</td>
<td>225.0</td>
<td>117.6</td>
</tr>
<tr>
<td>Deferred taxes (benefit)</td>
<td>(226.6)</td>
<td>(40.8)</td>
</tr>
<tr>
<td>Unrealized foreign currency loss (gain)</td>
<td>3.3</td>
<td>(4.1)</td>
</tr>
<tr>
<td>Unrealized (gain) loss on forward contracts</td>
<td>(11.5)</td>
<td>11.3</td>
</tr>
<tr>
<td>Unrealized gain on strategic equity investments</td>
<td>(25.8)</td>
<td>(9.6)</td>
</tr>
<tr>
<td>Inventory obsolescence charge</td>
<td>17.2</td>
<td>—</td>
</tr>
<tr>
<td>Impairment of intangible assets</td>
<td>2,053.3</td>
<td>—</td>
</tr>
<tr>
<td>Other</td>
<td>10.5</td>
<td>(2.3)</td>
</tr>
<tr>
<td>Changes in operating assets and liabilities, excluding the effect of acquisitions:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Accounts receivable</td>
<td>(137.6)</td>
<td>(186.4)</td>
</tr>
<tr>
<td>Inventories</td>
<td>(15.1)</td>
<td>(24.0)</td>
</tr>
<tr>
<td>Prepaid expenses, right of use operating assets and other assets</td>
<td>(54.8)</td>
<td>(126.8)</td>
</tr>
<tr>
<td>Accounts payable, accrued expenses, lease liabilities and other liabilities</td>
<td>(88.5)</td>
<td>23.6</td>
</tr>
<tr>
<td><strong>Net cash provided by operating activities</strong></td>
<td>1,339.6</td>
<td>995.3</td>
</tr>
<tr>
<td><strong>Cash flows from investing activities</strong>:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Purchases of available-for-sale debt securities</td>
<td>(15.4)</td>
<td>(41.4)</td>
</tr>
<tr>
<td>Proceeds from maturity or sale of available-for-sale debt securities</td>
<td>166.3</td>
<td>139.3</td>
</tr>
<tr>
<td>Purchases of mutual funds related to nonqualified deferred compensation plan</td>
<td>(9.8)</td>
<td>(10.9)</td>
</tr>
<tr>
<td>Proceeds from sale of mutual funds related to nonqualified deferred compensation plan</td>
<td>5.3</td>
<td>9.0</td>
</tr>
<tr>
<td>Purchases of property, plant and equipment</td>
<td>(19.4)</td>
<td>(92.8)</td>
</tr>
<tr>
<td>Payment for acquisition of business, net of cash acquired</td>
<td>(837.7)</td>
<td>—</td>
</tr>
<tr>
<td>Purchases of strategic equity investments and options</td>
<td>(38.1)</td>
<td>(43.8)</td>
</tr>
<tr>
<td>Purchase of intangible assets</td>
<td>—</td>
<td>(8.0)</td>
</tr>
<tr>
<td>Other</td>
<td>—</td>
<td>0.2</td>
</tr>
<tr>
<td><strong>Net cash used in investing activities</strong></td>
<td>(754.5)</td>
<td>(38.1)</td>
</tr>
<tr>
<td><strong>Cash flows from financing activities</strong>:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Payments on term loan</td>
<td>(65.3)</td>
<td>(32.7)</td>
</tr>
<tr>
<td>Payments as evolving credit facility</td>
<td>—</td>
<td>(250.0)</td>
</tr>
<tr>
<td>Repurchases of common stock</td>
<td>(360.8)</td>
<td>(48.9)</td>
</tr>
<tr>
<td>Net proceeds from issuance of common stock under share-based compensation arrangements</td>
<td>12.9</td>
<td>20.5</td>
</tr>
<tr>
<td>Other</td>
<td>(17.5)</td>
<td>(2.4)</td>
</tr>
<tr>
<td><strong>Net cash used in financing activities</strong></td>
<td>(430.7)</td>
<td>(313.5)</td>
</tr>
<tr>
<td>Effect of exchange rate changes on cash and cash equivalents and restricted cash</td>
<td>(8.1)</td>
<td>0.7</td>
</tr>
<tr>
<td>Net change in cash and cash equivalents and restricted cash</td>
<td>149.3</td>
<td>61.7</td>
</tr>
<tr>
<td>Cash and cash equivalents and restricted cash at beginning of period</td>
<td>2,729.6</td>
<td>1,367.3</td>
</tr>
<tr>
<td><strong>Cash and cash equivalents and restricted cash at end of period</strong></td>
<td>$ 2,879.6</td>
<td>$ 1,084.7</td>
</tr>
</tbody>
</table>
### Reconciliation of GAAP to non-GAAP R&D Expense

<table>
<thead>
<tr>
<th></th>
<th>2017</th>
<th>2018</th>
<th>2019</th>
<th>2020</th>
</tr>
</thead>
<tbody>
<tr>
<td>GAAP R&amp;D Expense</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>736</td>
<td>646</td>
<td>721</td>
<td>948</td>
</tr>
<tr>
<td>Upfront and milestone payments related to licenses and collaborations</td>
<td>76</td>
<td>57</td>
<td>62</td>
<td>92</td>
</tr>
<tr>
<td>Restructuring related expenses</td>
<td>49</td>
<td>27</td>
<td>103</td>
<td>-</td>
</tr>
<tr>
<td>Non-GAAP R&amp;D Expense</td>
<td>16</td>
<td>0</td>
<td>-</td>
<td>-</td>
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</tbody>
</table>

<table>
<thead>
<tr>
<th></th>
<th>2017</th>
<th>2018</th>
<th>2019</th>
<th>2020</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>878</td>
<td>730</td>
<td>886</td>
<td>1,040</td>
</tr>
</tbody>
</table>
### Reconciliation of GAAP to non-GAAP EPS

<table>
<thead>
<tr>
<th>Year</th>
<th>GAAP Net Income</th>
<th>GAAP Earnings per Common Share - Diluted</th>
<th>Non-GAAP Net Income</th>
<th>Non-GAAP Earnings per Common Share - Diluted</th>
</tr>
</thead>
<tbody>
<tr>
<td>2016</td>
<td>$399.4</td>
<td>$1.76</td>
<td>$1,054.4</td>
<td>$4.62</td>
</tr>
<tr>
<td>2017</td>
<td>$443.3</td>
<td>$1.97</td>
<td>$1,337.5</td>
<td>$5.86</td>
</tr>
<tr>
<td>2018</td>
<td>$77.6</td>
<td>$0.35</td>
<td>$1,798.6</td>
<td>$7.92</td>
</tr>
<tr>
<td>2019</td>
<td>$2,404.3</td>
<td>$10.70</td>
<td>$2,397.3</td>
<td>$10.53</td>
</tr>
<tr>
<td>2020</td>
<td>$252.0</td>
<td>$1.13</td>
<td>$2,435.5</td>
<td>$10.80</td>
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</table>

#### GAAP net income

<table>
<thead>
<tr>
<th>Year</th>
<th>2016</th>
<th>2017</th>
<th>2018</th>
<th>2019</th>
<th>2020</th>
</tr>
</thead>
<tbody>
<tr>
<td>Before tax adjustments:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>11.1</td>
<td>11.1</td>
<td>16.0</td>
<td>14.2</td>
<td>16.0</td>
</tr>
<tr>
<td>Fair value adjustment in inventory acquired</td>
<td>10.8</td>
<td>5.2</td>
<td>-</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Restructuring related expenses</td>
<td>-</td>
<td>152.1</td>
<td>5.8</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Research and development expense:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>57.6</td>
<td>76.4</td>
<td>57.4</td>
<td>61.7</td>
<td>92.5</td>
</tr>
<tr>
<td>Upfront and milestone payments related to licenses and other strategic agreements</td>
<td>9.6</td>
<td>49.4</td>
<td>26.7</td>
<td>103.4</td>
<td>-</td>
</tr>
<tr>
<td>Restructuring related expenses</td>
<td>-</td>
<td>16.3</td>
<td>0.1</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Selling, general and administrative expense:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>123.7</td>
<td>155.7</td>
<td>129.6</td>
<td>161.1</td>
<td>180.0</td>
</tr>
<tr>
<td>Restructuring related expenses</td>
<td>-</td>
<td>10.9</td>
<td>19.4</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Litigation charges</td>
<td>-</td>
<td>-</td>
<td>13.0</td>
<td>0.1</td>
<td>22.0</td>
</tr>
<tr>
<td>Gain on sale of asset</td>
<td>-</td>
<td>-</td>
<td>(3.5)</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Acquired in-process research and development</td>
<td>-</td>
<td>-</td>
<td>1,183.0</td>
<td>(4.1)</td>
<td>-</td>
</tr>
<tr>
<td>Amortization of purchased intangible assets</td>
<td>322.2</td>
<td>320.1</td>
<td>320.1</td>
<td>309.6</td>
<td>202.0</td>
</tr>
<tr>
<td>Change in fair value of contingent consideration</td>
<td>35.7</td>
<td>41.0</td>
<td>116.5</td>
<td>11.6</td>
<td>31.0</td>
</tr>
<tr>
<td>Acquisition-related costs</td>
<td>2.3</td>
<td>-</td>
<td>-</td>
<td>-</td>
<td>131.0</td>
</tr>
<tr>
<td>Restructuring expenses</td>
<td>3.0</td>
<td>104.6</td>
<td>25.5</td>
<td>12.0</td>
<td>(1.0)</td>
</tr>
<tr>
<td>Impairment of intangible assets</td>
<td>85.0</td>
<td>31.0</td>
<td>-</td>
<td>-</td>
<td>2,053.0</td>
</tr>
<tr>
<td>Investment income and (expense):</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>(Gains) and losses related to strategic equity investments</td>
<td>-</td>
<td>-</td>
<td>(43.1)</td>
<td>(59.7)</td>
<td>(26.0)</td>
</tr>
<tr>
<td>Other income and (expense):</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Gain related to purchase option</td>
<td>-</td>
<td>-</td>
<td>-</td>
<td>(32.0)</td>
<td>-</td>
</tr>
<tr>
<td>Restructuring related expenses</td>
<td>-</td>
<td>2.6</td>
<td>(0.1)</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Adjustments to income tax expense</td>
<td>(6.0)</td>
<td>(82.2)</td>
<td>(145.4)</td>
<td>(584.9)</td>
<td>(517.0)</td>
</tr>
</tbody>
</table>

#### Non-GAAP net income

$1,054.4 $1,337.5 $1,798.6 $2,397.3 $2,435.5

#### Shares used in computing diluted earnings per common share

<table>
<thead>
<tr>
<th>Year</th>
<th>GAAP</th>
<th>Non-GAAP</th>
</tr>
</thead>
<tbody>
<tr>
<td>2016</td>
<td>226.3</td>
<td>228.3</td>
</tr>
<tr>
<td>2017</td>
<td>225.4</td>
<td>228.1</td>
</tr>
<tr>
<td>2018</td>
<td>224.5</td>
<td>227.1</td>
</tr>
<tr>
<td>2019</td>
<td>224.8</td>
<td>227.6</td>
</tr>
<tr>
<td>2020</td>
<td>222.5</td>
<td>225.5</td>
</tr>
<tr>
<td>Reconciliation of GAAP to non-GAAP Operating Margin</td>
<td>2017</td>
<td>2018</td>
</tr>
<tr>
<td>--------------------------------------------------</td>
<td>------</td>
<td>------</td>
</tr>
<tr>
<td>GAAP operating margin (% of total revenues)</td>
<td>18%</td>
<td>7%</td>
</tr>
<tr>
<td>Share-based compensation</td>
<td>7%</td>
<td>5%</td>
</tr>
<tr>
<td>Amortization of purchased intangible assets</td>
<td>9%</td>
<td>8%</td>
</tr>
<tr>
<td>Change in fair value of contingent consideration</td>
<td>1%</td>
<td>3%</td>
</tr>
<tr>
<td>Upfront payments related to licenses and other strategic agreements</td>
<td>1%</td>
<td>1%</td>
</tr>
<tr>
<td>Contingent milestone payments</td>
<td>0%</td>
<td>0%</td>
</tr>
<tr>
<td>Acquired in-process research and development</td>
<td>0%</td>
<td>29%</td>
</tr>
<tr>
<td>Acquisition-related cost</td>
<td>0%</td>
<td>0%</td>
</tr>
<tr>
<td>Restructuring expenses</td>
<td>8%</td>
<td>1%</td>
</tr>
<tr>
<td>Litigation charges</td>
<td>0%</td>
<td>0%</td>
</tr>
<tr>
<td>Gain on sale of asset</td>
<td>0%</td>
<td>0%</td>
</tr>
<tr>
<td>Impairment of intangible assets</td>
<td>1%</td>
<td>0%</td>
</tr>
<tr>
<td>Non-GAAP operating margin (% of total revenues)</td>
<td>45%</td>
<td>53%</td>
</tr>
</tbody>
</table>