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Chief Executive Officer
37th Annual
J.P. Morgan Healthcare Conference
January 8, 2019



Forward-Looking Statements

This presentation contains forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995, including statements related to: a growing and diversifying product portfolio; there are four durable, growing blockbuster franchises; ALXN1840 (WTX1010) is a potential growth driver; our ambition to continue to deliver double digit revenue growth; Soliris patients will continue to expand; potential future regulatory approval for Soliris as a treatment for NMOSD (and timing of such approval); underlying momentum builds potential strong platform for Soliris launch in NMOSD; planned timing for the initiation of clinical trials and the timing of expected receipt/release of results of clinical trials; opportunity to expand in complement with CP010 addressing neurological disorders; the Dicerna transaction provides the potential for multiple targets in complement; Company future plans to further rebuild the product pipeline through business development; all five of the Company's 2019 key objectives; Ultomiris has the potential to become a new standard of care for PNH; goal of converting 70% of PNH patients from Soliris to Ultomiris in two years in the US (and confidence in ability to facilitate such rapid conversion); Ultomiris has a globally sustainable pricing strategy; all aspects of the ALXN1210 future development programs, including multiple formulations could provide optionality for patients and to convert without interruption; future plans and timing for clinical trials and filing for regulatory approval for ALXN1210 development products (including IV and subcutaneous formulations and in gMG indication); plans to expand in neurology; future plans to pursue ALXN1210 for the treatment of ALS and PPMS and for future clinical trials related thereto (and the potential benefits of ALXN1210 in such indications); the future growth of the metabolic portfolio (including due to ALXN1840 and the potential benefits of ALXN1840); rare neurology portfolio presents significant growth opportunity; the Company has an emerging research portfolio and Alexion's future clinical, regulatory, and commercial plans for ALXN1210, ALXN1840 and other product candidates (and the potential benefits of such products). Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those forward-looking statements, including for example: our dependence on sales from our principal product (Soliris); our ability to timely switch PNH patients (and any future indications) from Soliris to Ultomiris; payer, physician and patient acceptance of Ultomiris as an alternative to Soliris; appropriate pricing for Ultomiris; future competition from biosimilars and other products; decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products; delays or failure of product candidates (including ALXN1210 developmental products, ALXN1840, Soliris for NMOSD and ALXN1210 for ALS and PPMS) to obtain regulatory approval; delays or the inability to launch product candidates (including ALXN1210 and ALXN1840) due to regulatory restrictions, anticipated expense or other matters; interruptions or failures in the manufacture and supply of our products and our product candidates; failure to satisfactorily address matters raised by the FDA and other regulatory agencies; results in early stage clinical trials may not be indicative of full results or results from later stage or larger clinical trials (or broader patient populations) and do not ensure regulatory approval; the possibility that results of clinical trials are not predictive of safety and efficacy and potency of our products (or we fail to adequately operate or manage our clinical trials) which could cause us to halt trials, delay or prevent us from making regulatory approval filings or result in denial of approval of our product candidates (including ALXN1210 as a treatment for ALS and PPMS); unexpected delays in clinical trials; unexpected concerns that may arise from additional data or analysis obtained during clinical trials; future product improvements may not be realized due to expense or feasibility; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; inability to complete planned acquisitions due to failure of regulatory approval or material changes in target or otherwise; inability to complete acquisitions and investments due to increased competition for technology; the possibility that current rates of adoption of Soliris® in PNH, aHUS, gMG or other diseases are not sustained; the adequacy of our pharmacovigilance and drug safety reporting processes; failure to protect and enforce our data, intellectual property and proprietary rights and the risks and uncertainties relating to intellectual property claims and challenges against us (including infringement suits against Ultomiris); the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all; failure to realize the benefits and potential of investments, collaborations, licenses and acquisitions; the possibility that expected tax benefits will not be realized; delay of collection or reduction in reimbursement due to adverse economic conditions or changes in government and private insurer regulations and approaches to reimbursement; uncertainties surrounding legal proceedings, company investigations and government investigations, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice; the risk that estimates regarding the number of patients with PNH, aHUS, gMG, HPP and LAL-D and other future indications we are pursuing are inaccurate; the risks of changing foreign exchange rates; risks relating to the potential effects of the Company's restructuring; and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2018 and in our other filings with the SEC. Alexion disclaims any obligation to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

In addition to financial information prepared in accordance with GAAP, this presentation also contains non-GAAP financial measures that Alexion believes, when considered together with the GAAP information, provide investors and management with supplemental information relating to performance, trends and prospects that promote a more complete understanding of our operating results and financial position during different periods. For information on what is excluded from the non-GAAP information, see ir.alexion.com. These non-GAAP financial measures are not intended to be considered in isolation or as a substitute for, or superior to, the financial measures prepared and presented in accordance with GAAP, and should be reviewed in conjunction with the relevant GAAP financial measures. Please refer to ir.alexion.com for reconciliations of GAAP to non-GAAP financial results presented herein.



Our Mission

Serving patients and their families is our unwavering mission – they are our guiding star and they inspire us to continue to find answers.

We act with integrity, urgency, and discipline because we know that lives are at stake.

Our Focus is on Rare Diseases

>7,000
rare diseases
identified

Only 500 rare diseases
have approved therapies

~30M patients
diagnosed in US,
50% are children

Four transformative therapies across five rare diseases in our growing portfolio



Paroxysmal Nocturnal
Hemoglobinuria (PNH)

Prior to SOLIRIS®, up to
35% of patients with
available support did not
survive beyond 5 years



Atypical hemolytic anemia
(aHUS)

Prior to SOLIRIS®, 79% of
patients died, required kidney
dialysis, or had permanent
kidney damage within three
years of diagnosis



Generalized Myasthenia
Gravis (gMG)

Debilitating, chronic and
progressive autoimmune
neuromuscular disease that
can lead to inability to walk,
talk, eat or breathe



Hypophosphatasia (HPP)

Prior to STRENSIQ®, children
with symptoms prior to 6
months of age had 73%
mortality rate at 5 years



Lysosomal Acid Lipase
Deficiency (LAL-D)

Prior to KANUMA®, Median
age of death was 3.7 months
in infants with rapid disease
progression



Source: Caprioli J, Noris M, Brioschi S, et al. The impact of MCP, CFH, and IF mutations on clinical presentation, response to treatment, and outcome. *Blood*. 2006;108:1267-1269. Noris M, Caprioli J, Bresin E, et al. Relative Role of Genetic Complement Abnormalities in Sporadic and Familial aHUS and Their Impact on Clinical Phenotype. *CJASN*. 2010;5:1844-1859. Hillmen P, Lewis SM, Bessler M, Luzzatto L, Dacie JV. Natural history of paroxysmal nocturnal hemoglobinuria. *N Engl J Med*. 1995 Nov 9;333(19):1253-8. Whyte MP, Leung E, Wilcox W, et al. Hypophosphatasia: a retrospective natural history study of the severe perinatal and infantile forms. Poster presented at the 2014 Pediatric Academic Societies and Asian Society for Pediatric Research Joint Meeting, Vancouver, B.C., Canada, May 5, 2014. Abstract 752416. Jones SA, Valayannopoulos V, Schneider E, et al. Rapid progression and mortality of lysosomal acid lipase deficiency presenting in infants. *Genet Med*. 2016;18(5):452-458. doi: 10.1038/gim.2015.108

Provided January 8, 2019, as part of an oral presentation and is qualified by such, contains forward-looking statements, actual results may vary materially; Alexion disclaims any duty to update.



2018 in Review

Strengthened Our Foundation

Reinforced Culture of Compliance

Strengthened Leadership Team

Refreshed Board of Directors

Refocused Corporate Strategy

Restructured to Optimize Organization and
Resource Allocation



Delivered on our 2018 Objectives

- 1

Grow In-line Business

✓ Strong top-line execution in both complement and metabolic portfolios
- 2

Drive SOLIRIS® Launch in gMG

✓ Best SOLIRIS® launch to date
✓ 788 patients on therapy as of December 31, 2018
- 3

Extend Complement Leadership with ULTOMIRIS™

✓ Positive Phase 3 results in PNH naïve and switch patients
✓ Early FDA approval December 2018
- 4

Advance and Rebuild the Pipeline

✓ Positive Phase 3 results in NMOSD
✓ Acquired two clinical stage assets*
✓ Announced two collaborations†
- 5

Deliver on Financial Ambitions

✓ Double-digit revenue and non-GAAP EPS growth through 3Q18‡
✓ Non-GAAP operating margin above 50%



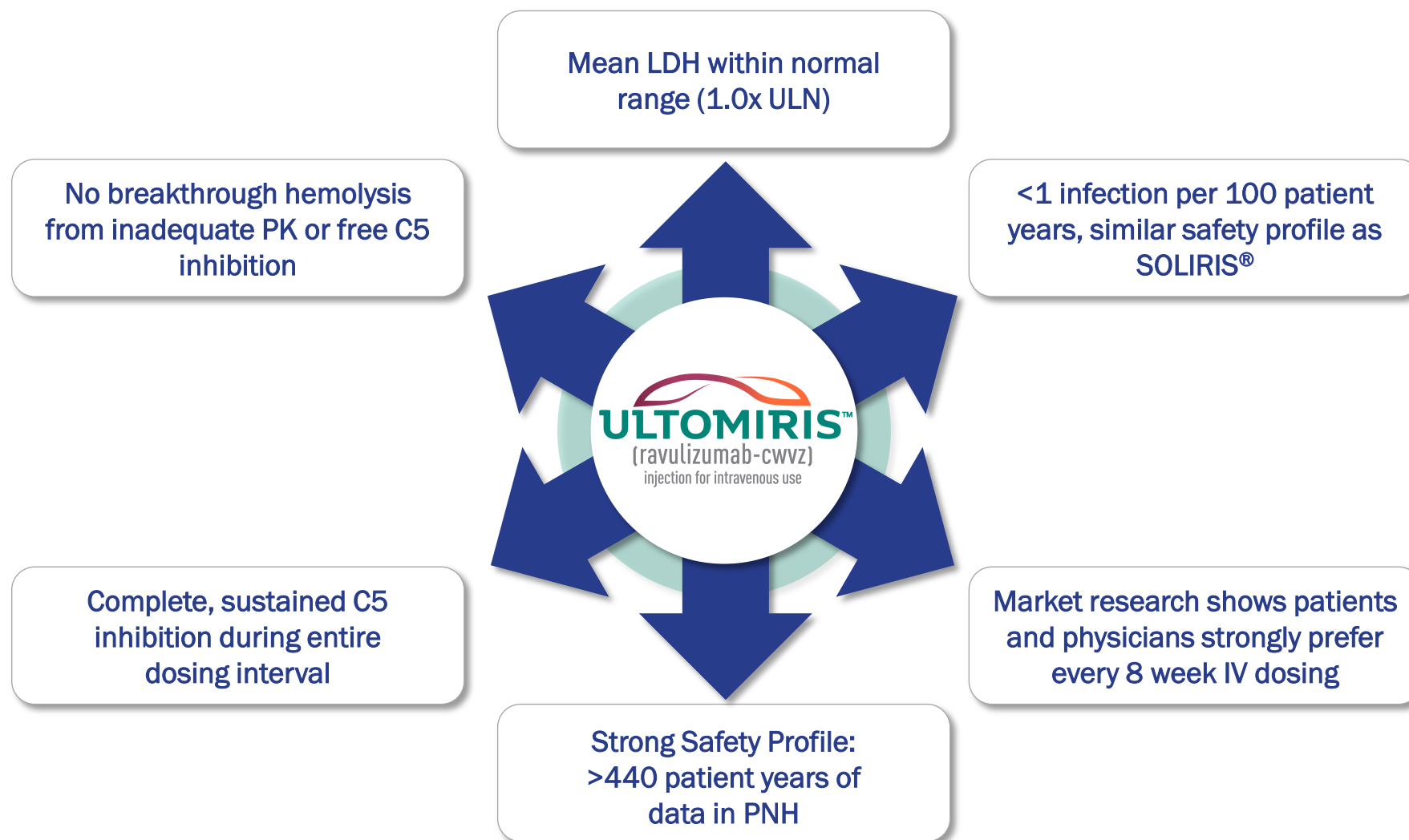
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*Wilson Therapeutics and Syntimmune Acquisitions

†Complement Pharma and Dicerna Pharmaceuticals

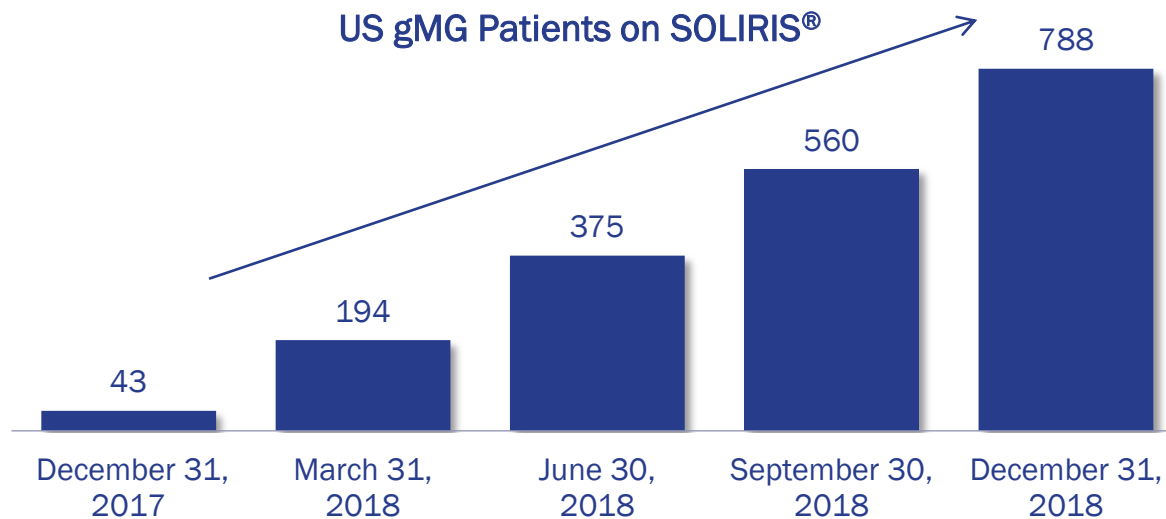
‡Reconciliation of adjusted non-GAAP measures to GAAP measures can be found on ir.alexion.com

ULTOMIRIS™: Clear, Compelling Value Proposition



gMG is the Best Alexion Launch to Date

- Continue to expand our family of SOLIRIS® patients
- Underlying momentum builds potential strong platform for SOLIRIS® launch in NMOSD*



Note: gMG = generalized myasthenia gravis

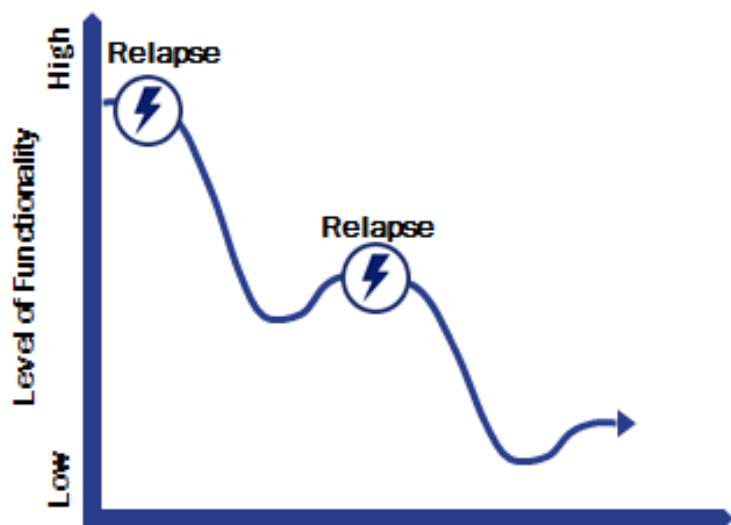
*SOLIRIS® not yet approved in NMOSD, sBLA filing submitted

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Remarkable Phase 3 SOLIRIS® Results in NMOSD

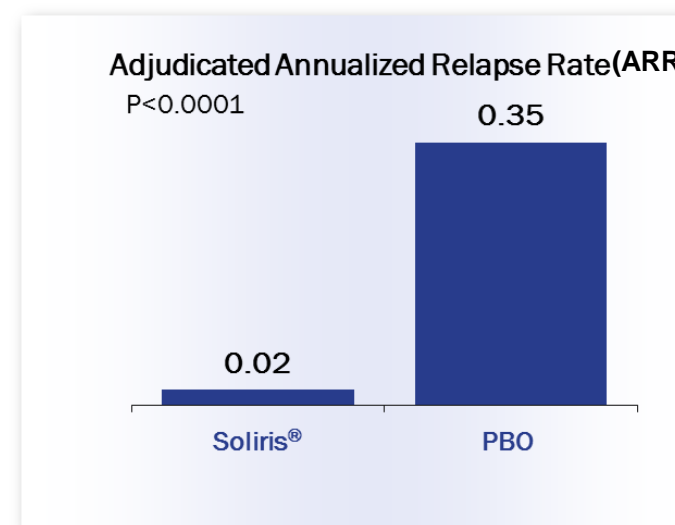
97.9% of SOLIRIS® patients relapse-free at 48 weeks vs. 63.2% for placebo

NMOSD is characterized by step-wise deterioration following each relapse



Relapses can lead to cognitive worsening, encephalopathy, seizures, pain, paralysis, and vision loss , blindness or death

Significant reduction in adjudicated ARR with SOLIRIS® on top of SOC †



94.2% Reduction in Risk for Relapse (Hazard Ratio of 0.058); $p < 0.0001$

Strong Business Development Execution

- ALXN1840 addresses significant unmet need in Wilson Disease
- Now powering Phase 3 study for superiority



Announced April 11, 2018



Announced June 11, 2018

- Preclinical C6 program underway
- Opportunity to expand in complement with a novel asset (CP010) addressing neurological disorders

- ALXN1830 showed proof of mechanism in PV/PF Phase 1b study
- Leading anti-FcRn in development for WAIHA
- Plan to initiate two pivotal studies in 2019



Announced September 26, 2018



Announced October 24, 2018

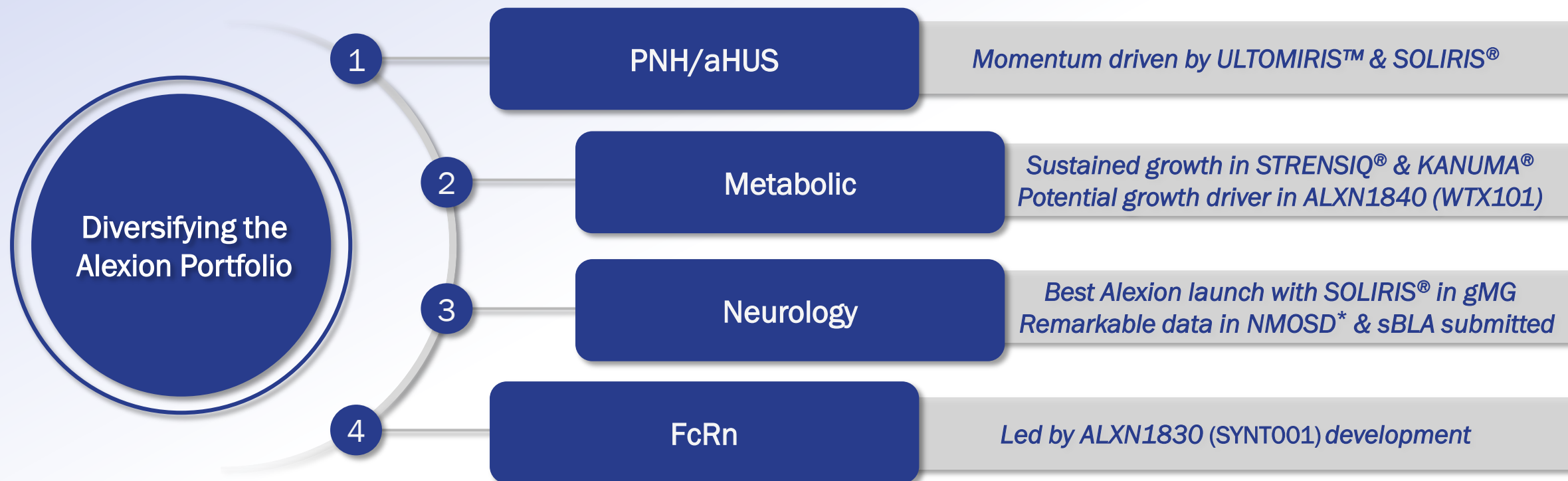
- Preclinical RNAi programs underway
- Innovative technology platform, potential for multiple targets in complement

Maintain capacity to further rebuild the pipeline through disciplined and strategic business development



Our Vision for the Future

Building Four Durable, Growing Blockbuster Franchises



Financial Ambition to Continue to Deliver Double-Digit Revenue Growth

*NMOSD = Neuromyelitis Optica Spectrum Disorder

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Continuing our Momentum into 2019

Our 2019 Key Objectives

- 1 ULTOMIRIS™ Conversion in PNH; ALXN1210 aHUS filing
- 2 Accelerate Neurology Portfolio
- 3 Grow our Metabolic Portfolio
- 4 Execute and Expand the Pipeline
- 5 Deliver on Financial Ambitions

Maintain our Core Values to Serve Patients while Acting with Integrity

ULTOMIRIS™ Approved and Launched



ULTOMIRIS™: Potential to Become A New Standard of Care for PNH

Confident in Ability to Facilitate Rapid Conversion to ULTOMIRIS

**Patient Preferred
Dosing Profile**

**Globally
Sustainable
Pricing Strategy**

**Patients Can
Safely Switch to
ULTOMIRIS from
SOLIRIS®**

11+

**Years of
Commercial
Experience**

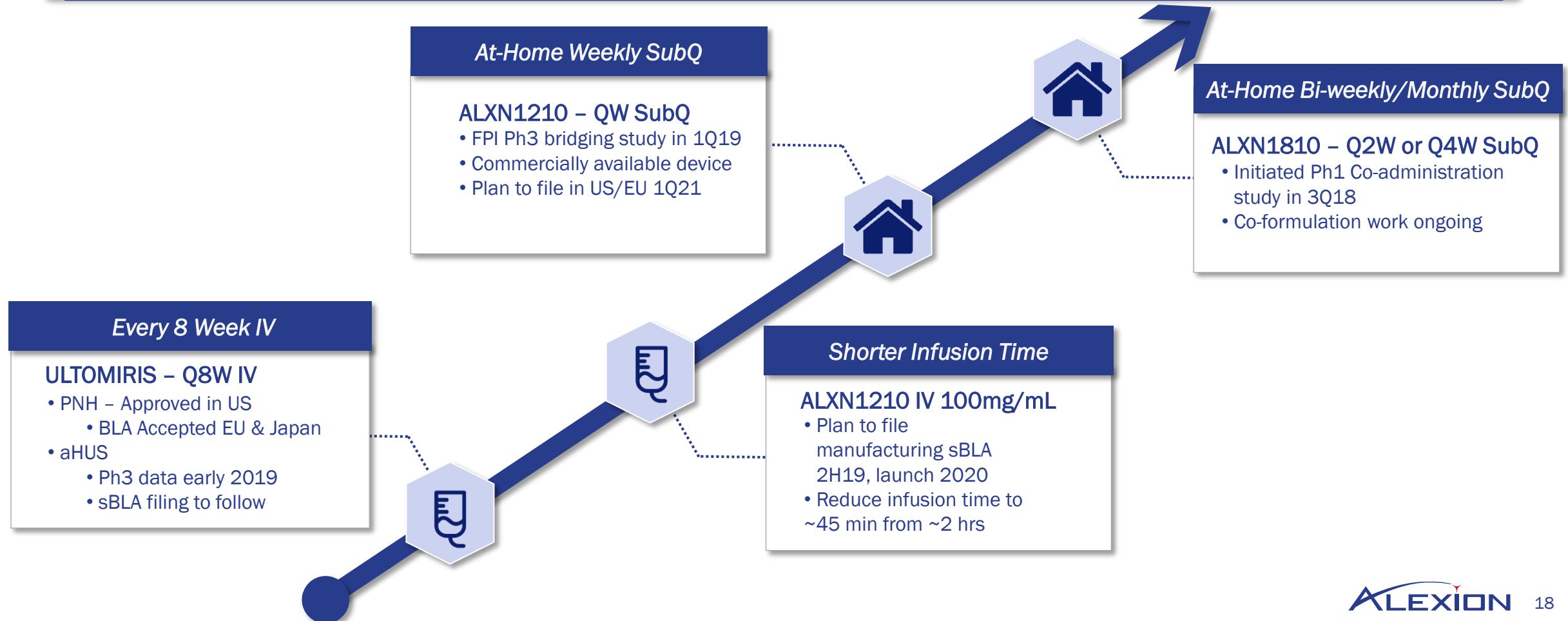
Conversion Goal of >70% in First Two Years of Launch in US

Advancing ALXN1210 Development Programs

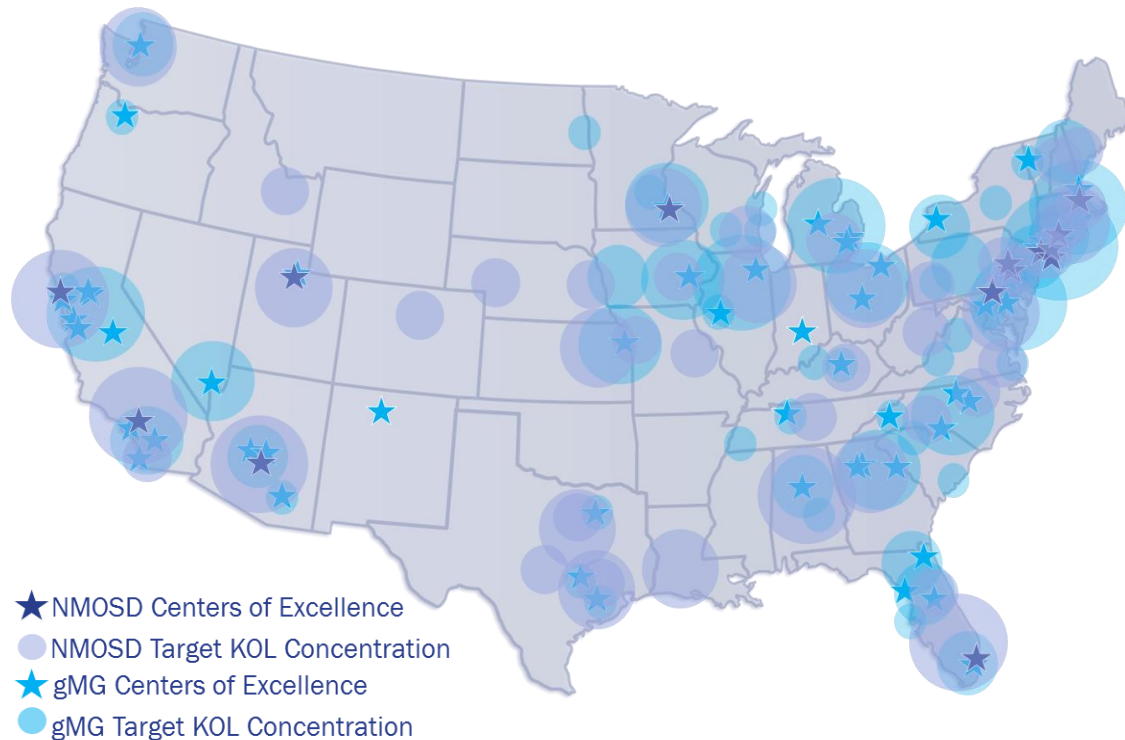
Raising the Bar & Expanding Treatment Options

Potential First Approved
SubQ C5 inhibitor

Multiple formulations could provide optionality for patients & ability to convert without interruption



Building on Our Rare Disease Expertise to Expand in Neurology



Leverage and expand existing Neurology sales force, OneSource™, Medical, Payer teams

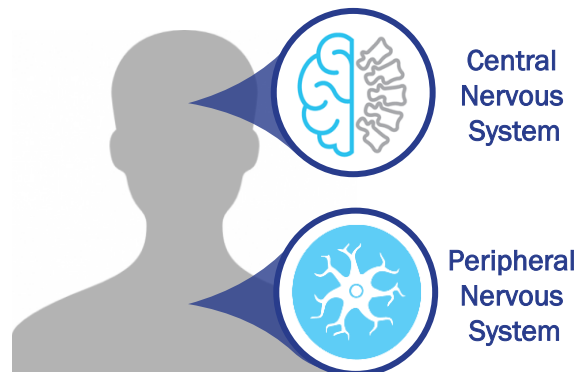
gMG

- More than 30% of prescribing physicians have treated more than one patient
- Plan to initiate Phase 3 study of ALXN1210 in gMG in early 2019

NMOSD

- SOLIRIS® NMOSD sBLA filing submitted in US
 - Launch prep underway, anticipate approval in 2H19
- Planning to submit regulatory filings in EU and Japan
- Plan to initiate Phase 3 study of ALXN1210 in NMOSD in 2H19

Advancing our Neurology Pipeline with ALXN1210



Complement confirmed to play a key role in both the CNS and Neuromuscular Junction with SOLIRIS® in MG and NMOSD

Scientific rationale supports potential role of complement, including MAC deposition in ALS and C5b-9 elevation prior to relapse in PPMS patients

Amyotrophic Lateral Sclerosis (ALS)

- Neurodegenerative disease characterized by progressive muscular paralysis due to motor neuron degeneration
- High mortality rate 3-5 years post-diagnosis
- Estimated 15-20K addressable population in US, EU5, and Japan
- Plan to initiate POC clinical trial in 2019

Primary Progressive Multiple Sclerosis (PPMS)

- Progressive, worsening neurologic disease characterized by decreased mobility, functional impairment, cognitive changes
- Estimated 30-40K addressable population in US, EU5, and Japan
- 15% of MS patients diagnosed with PPMS
- Plan to initiate POC clinical trial in 2019

Growing Metabolics Portfolio



STRENSIQ®

- Continuing to identify new patients
- Launched and reimbursed in 7 countries
- Ongoing work to secure additional agreements

KANUMA®

- Continuing to identify new patients with LAL-D
- Launched and reimbursed in 8 countries
- Improving funding agreements and increasing access

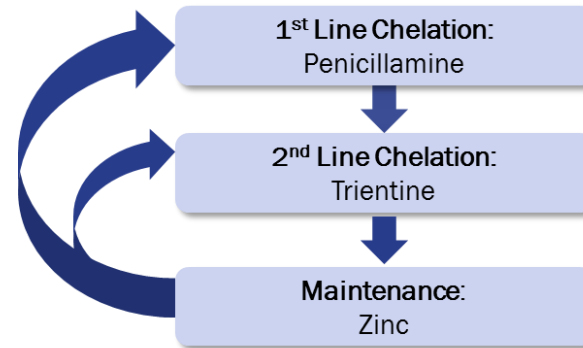


Further Growth in Metabolics with ALXN1840 (WTX101) in Wilson disease

Ongoing Phase 3 Trial
Powered for Superiority



Wilson disease poorly managed by current treatments, low compliance and potential for neurological worsening



Addressable Population

Prevalence 1 in 30,000
~10K in EU5 and ~10K in US

~50% Diagnosed and Treated
~5K in EU5 and ~5K in US

Differentiated product profile compared to currently available treatments



ALXN1830 (SYNT001): Initial Pivotal Trial in Warm Autoimmune Hemolytic Anemia (WAIHA)

WAIHA

No Currently Approved Therapy

Limited treatment options

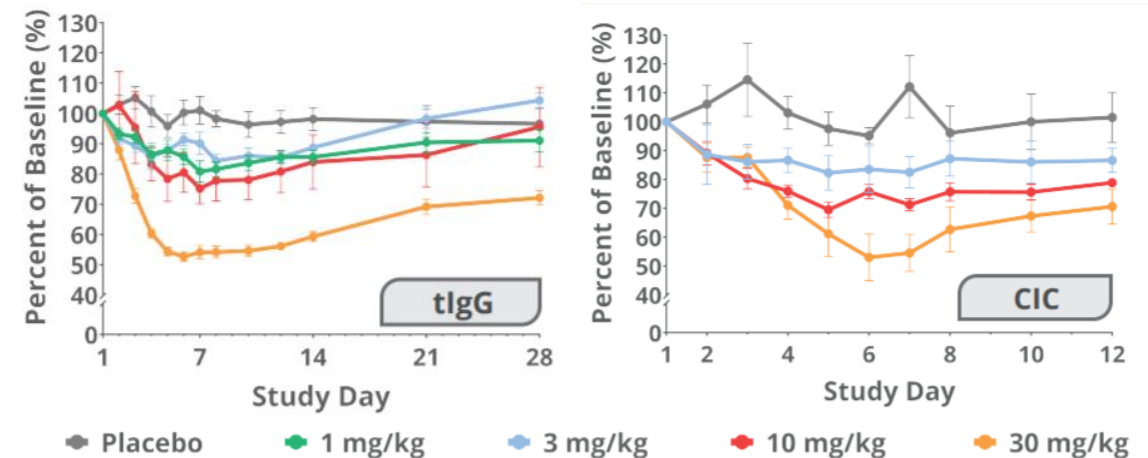
~65K Patients in US and EU5

~1/3 of patients have active disease despite corticosteroids and IST treatment

Significant Unmet Need for Rapid Onset, Disease Control & Improved Safety Profile

Complications include weakness, heart failure, hepatomegaly and splenic enlargement

ALXN1830 Induced Rapid Clearance of Monomeric and Multimeric IgG in a Dose-Dependent Manner



Source: SYNT001: a Humanized IgG4 Monoclonal Antibody that Disrupts the Interaction of FcRn & IgG for the Treatment of IgG-Mediated Autoimmune Diseases Laurence Blumberg, MDa ; John E. Humphries, MDb; Kenneth C. Lasseter, MDc ; Richard S. Blumberg, MDd a Syntimmune, Inc., New York, NY; bBiologics Consulting, Alexandria, VA; c Clinical Pharmacology of Miami, Inc., Miami, FL; dBrigham and Women's Hospital, Boston, MA

ALXN1830 is the first, and only, anti-FcRn currently in development for WAIHA

Evolving to Drive Long-term Value Creation



- Strengthened corporate foundation, strong business momentum heading into 2019
- Confidence in rapid conversion to ULTOMIRIS, given strong value proposition and capabilities
- Building four durable, growing blockbuster franchises
- Rare neurology portfolio including gMG and potentially NMOSD presents significant growth opportunity
- Continuing to build the pipeline with internal assets and disciplined business development
- Complement leadership and emerging research portfolio



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Q&A

