Alexion to Acquire Achillion

Conference Call
October 16, 2019

Introduction	Susan Altschuller, Ph.D., Vice President, Investor Relations
Strategic Rationale	Ludwig Hantson, Ph.D., Chief Executive Officer
Financial Overview & Lead Indications	Aradhana Sarin, M.D., Chief Strategy and Business Officer
Overview of Factor D Portfolio	John Orloff, M.D., Head of R&D
Closing Remarks	Ludwig Hantson, Ph.D., Chief Executive Officer
Available for Q&A	Brian Goff, Chief Commercial Officer and Paul Clancy, CFO

FORWARD LOOKING STATEMENTS

This presentation contains forward-looking statements including, statements related to: the proposed acquisition of Achillion by Alexion; Alexion's ability to create value for patients and shareholders from the acquisition of Achillion and Alexion's ability to advance Achillion's pipeline; Achillion's lead candidate danicopan's ability to enhance treatment for PNH patients experiencing extravascular hemolysis (EVH) and, as a combination therapy, to establish a new standard of care for these patients; therapeutic benefits of Achillion products, including potential first-in-class C3 glomerulopathy (C3G) and Factor D inhibitors; development and regulatory timelines for ACH-4471 and ACH-5228 and Phase 2 C3G studies; Alexion's ability to build and diversify in rare disease with the acquisition of Achillion; Alexion's ability to leverage its leading rare disease development and commercialization capabilities to further develop a portfolio of oral Factor D inhibitors; Achillion's platform will produce a platform for Factor D inhibition in additional alternative pathway complement-mediated rare diseases; Ahcillion's pipeline includes several small molecules that have the potential to treat immune-related diseases associated with the alternative pathway of the complement system; there is significant opportunity for Factor D inhibition in the treatment of diseases; the potential benefits of Alexion's transaction with Stealth BioThrepeutics (Stealth), the therapeutic benefits of Elamipretide to treat mitochondrial diseases and the anticipated clinical and regulatory timeline for the development of Elamipretide; and the anticipated closing date of the acquisition. A number of important factors could cause actual results to differ materially from those indicated by such forward-looking statements, including: the risk that the proposed acquisition of Achillion by Alexion may not be completed; the failure to receive the required stockholder approval necessary to complete the acquisition; the failure (or delay) to receive the required regulatory approvals of the proposed acquisition; the failure of the closing conditions set forth in the acquisition agreement to be satisfied (or waived); the anticipated benefits of the Achillion platform and therapies and Elamipretide may not be realized; future clinical trials of Achillion and Stealth products may not prove that the therapies are safe and effective to the level required by regulators; decisions of regulatory authorities regarding the adequacy of the research and clinical tests, marketing approval or material limitations on the marketing of Achillion and Stealth products; delays or failure of product candidates to obtain regulatory approval; delays or the inability to launch product candidates due to regulatory restrictions; anticipated expense or other matters; interruptions or failures in the manufacture and supply of products and product candidates; failure to satisfactorily address matters raised by the FDA and other regulatory agencies; the possibility that results of clinical trials are not predictive of safety and efficacy results of products in broader patient populations; the possibility that clinical trials of product candidates could be delayed or terminated prior to completion for a number of reasons; the adequacy of pharmacovigilance and drug safety reporting processes; 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, 2019 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.

Strategic Rationale

Ludwig Hantson, Ph.D.
Chief Executive Officer

Achillion Overview

- **Development-stage biopharmaceutical company**
  - Focused on developing transformative therapies for patients and families affected by diseases of the complement system
  - Based in New Haven, CT and Blue Bell, PA
  - Small molecule development expertise
- **Current pipeline includes two clinical stage oral Factor D inhibitors**
  - Lead candidate in Phase 2 for PNH patients experiencing clinical EVH and for patients with C3G
  - Next gen candidate completed successful Phase 1; Potential best-in-class oral Factor D inhibitor
- **Factor D is a critical control point for the complement system's alternative pathway (AP)**
  - Implicated in numerous rare diseases with significant unmet needs

Strategic Rationale

- ✓ **Opportunity to diversify into additional complement-mediated diseases using oral therapies**
  - Adds clinical-stage portfolio of oral small molecule Factor D inhibitors to Alexion pipeline
- ✓ **Potential to enhance treatment for PNH patients**
  - Opportunity to treat the small portion of PNH patients experiencing clinical extravascular hemolysis (EVH)
- ✓ **Potential first-in-class treatment for C3 glomerulopathy**
  - Severe kidney disease with no approved treatment
- ✓ **Promising development platform**
  - Significant opportunity for Factor D inhibition in other alternative pathway complement-mediated rare diseases
  - Small molecule chemistry expertise and library
- ✓ **Aligned with disciplined BD strategy**

Further builds diversified pipeline with potential to treat additional complement-mediated diseases, creating value for patients and shareholders

Financial Overview & Lead Indications

Aradhana Sarin, M.D.
Chief Strategy and Business Officer

ACQUISITION TERMS & FINANCIAL IMPACT

Deal Terms

Initial consideration of ~$930M or $6.30 per share

Also acquiring $230M* cash on Achillion's balance sheet

Potential additional consideration to be paid in the form of CVRs if certain clinical and regulatory milestones are achieved

Subject to approval by Achillion shareholders, approval from relevant regulatory agencies and other customary closing conditions

Financial Impact

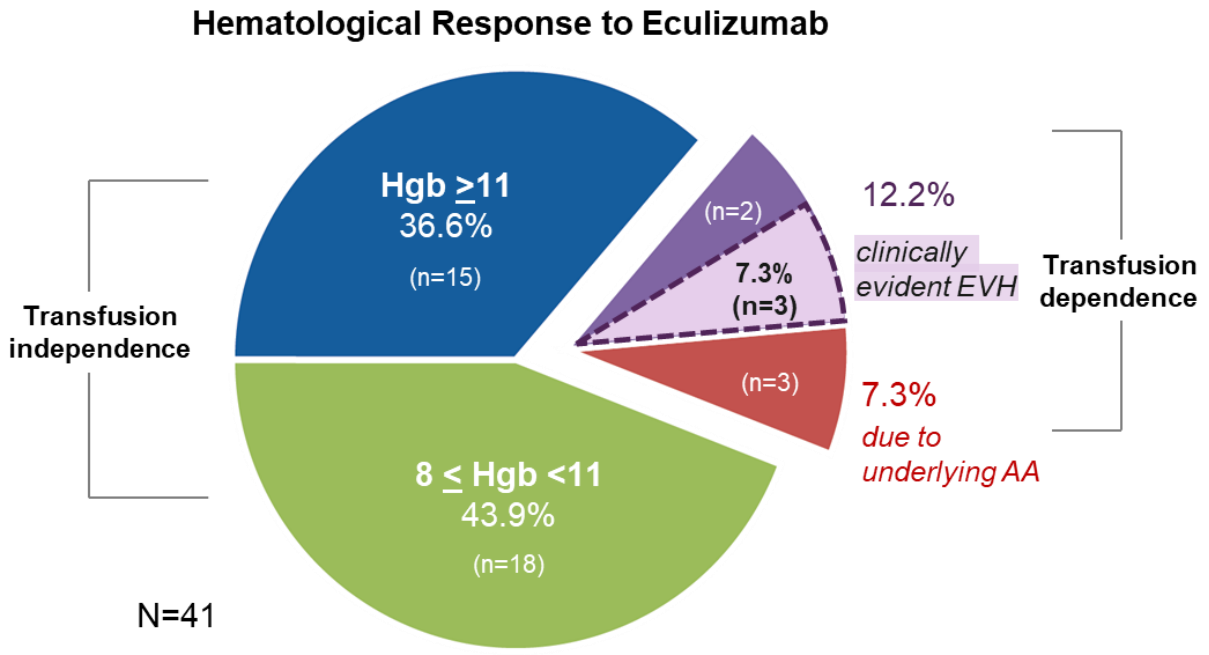
- Transaction will be financed with cash on hand
- 2019 financial impact covered as part of BD carve out included in prior guidance

Aligned With Our Disciplined Business Development Strategy

*Cash and marketable securities as of September 30th, 2019; Actual amount will be determined as of the transaction close

ENHANCING TREATMENT FOR PNH PATIENTS EXPERIENCING CLINICAL EVH

- **<10%** of C5-treated PNH patients experience clinical extravascular hemolysis (EVH)
- Achillion's lead compound **danicopan has demonstrated in clinical trials to work with C5 inhibition** to more effectively treat PNH patients with EVH
- Potential for combination therapy to **raise the standard of care** for this subset of PNH patients

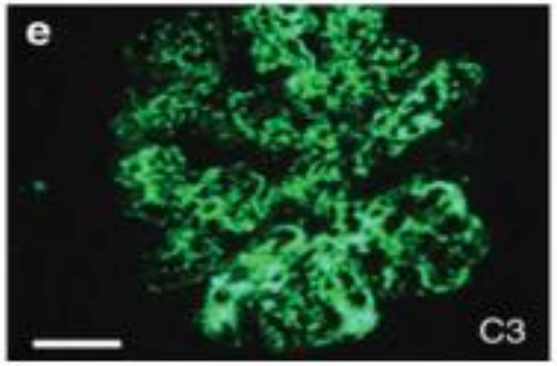


Clinically Evident EVH = Transfusion dependent + high reticulocytes + Coombs positive, without IVH (breakthrough hemolysis) or Bone Marrow Failure / Aplastic Anemia

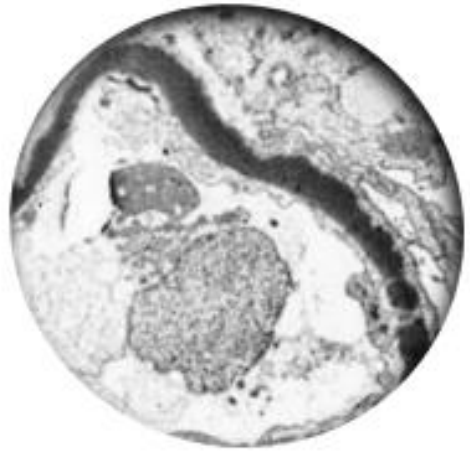
Hgb, hemoglobin.
Over 80% (33/41) of patients were transfusion-independent on eculizumab. ~17% (7/41) of patients had breakthrough IVH. All minor responders in this cohort were attributed to underlying AA
Partial responders included 5/41 (12%) patients. Patient #11 had breakthrough hemolysis due to IVH, and patient #38 had 5% C3 deposition, which was less than the median C3 deposition in the optimal responder group (~20% C3 deposition in this group). Therefore, clinically significant EVH in this cohort was found in 3/41 patients (7%), possibly also a result of underlying mild to moderate AA in these patients.
Risitano AM, et al. *Blood.* 2009;113(17):4094-4100.

POTENTIAL FIRST-IN-CLASS TREATMENT FOR C3G

- **Ultra-rare, chronic kidney disease** caused by uncontrolled complement activation and deposition
- Results in **devastating kidney damage**
  - Up to 50% of patients progress to kidney failure within 10 years
  - ~70% of C3G kidney transplant patients experience recurrence
- **No approved treatments** – efforts to slow process of kidney damage include corticosteroids, ISTs, ACEi/ARBs, dietary changes – **significant opportunity for new therapies** to target underlying cause
- Expect to **complete danicopan Phase 2 C3G studies** and target global regulatory discussions in 2020



Inappropriate and excessive consumption of C3 leads to an excess production of C3 fragments



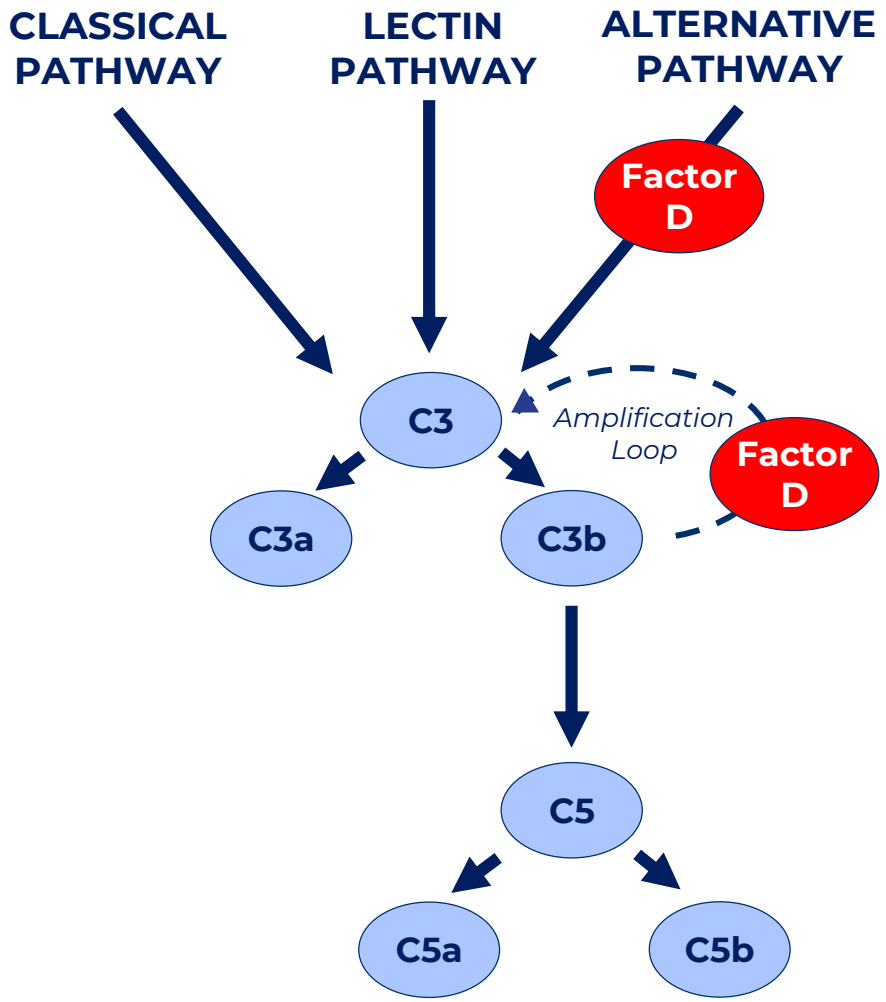
C3 fragments are deposited in kidney and may contribute to kidney damage

Overview of Factor D Portfolio

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

ACHILLION: TARGETING FACTOR D TO INHIBIT THE COMPLEMENT ALTERNATIVE PATHWAY

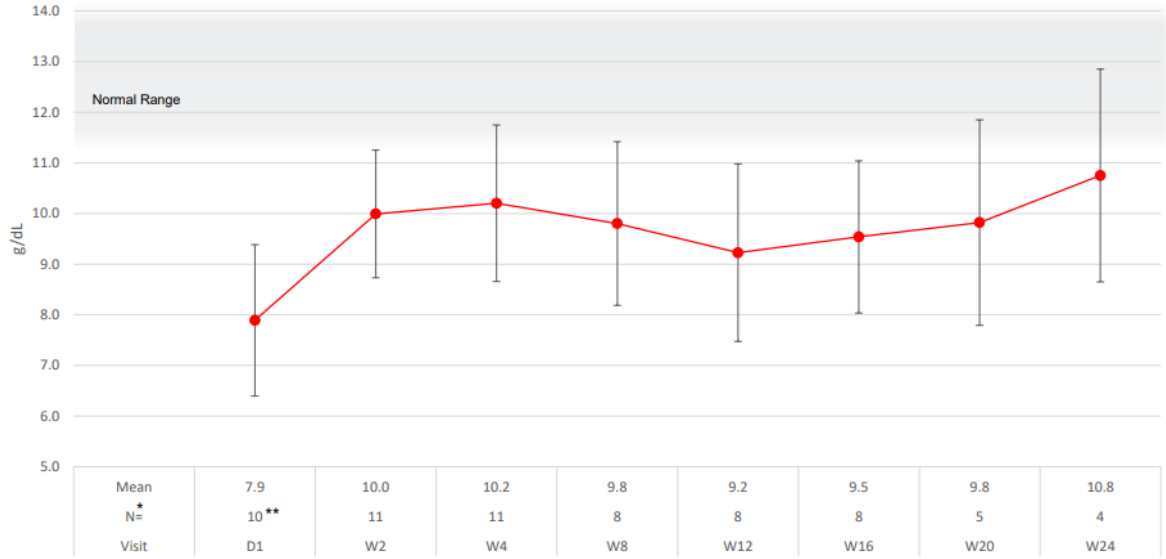
- **Targeting Factor D – an upstream regulator and a key, rate-limiting enzyme of the complement alternative pathway – allows for complement inhibition**
- **Selective inhibition of the alternative pathway leaves the classical pathway and lectin pathways intact to fight infections**
- **Strong rationale for Factor D inhibition in numerous complement-mediated rare diseases**
    - Commonality among complement-mediated renal and ophthalmology diseases is C3 activation / deposition
- **Factor D is targetable with small molecule inhibitors, allowing for oral route of administration**



DANICOPAN (ACH-4471): ADDRESSING PNH PATIENTS WITH EVH

- **Ongoing open-label, multi-dose Phase 2 study of danicopan TID (3x per day) + eculizumab in PNH patients with inadequate response to eculizumab:**
  - Red blood cell transfusion within the last 12 weeks
  - Anemia with adequate reticulocytosis
  - Stable regimen of eculizumab
- **Phase 2 interim data readout in May 2019 with significant mean hemoglobin increase from baseline to week 24 (n=4)**
  - Reduction in blood transfusions in the combination treatment period
- **Targeting Q4 2019 for full Phase 2 readout**
- **Plans to initiate Phase 3 combination study in PNH suboptimal responders in 2020 subject to regulatory feedback**

Phase 2 Interim Results: Hemoglobin Improvement Compared to Baseline (g/dL) (Danicopan + Eculizumab)



*Subjects with data available at each time point. Data as of May 10, 2019.
**Excludes Day 1 for 1 patient.

Danicopan for PNH has been granted Orphan Drug Designation in the US and the EU and FDA Breakthrough Designation

DANICOPAN (ACH-4471): FIRST POTENTIAL THERAPY FOR PATIENTS WITH C3G

- **Positive signals for reduction of AP hyperactivity in alternative pathway biomarkers**
- **14-day monotherapy proof of mechanism trial (n=6) validated danicopan's impact to serum and urine complement biomarkers; No SAEs**

Biomarker (units)	Targeted Outcome	Outcome (n=6)
Serum C3 (g/L)	↑	4 of 6 patients higher Serum C3 ✓
Bb (ug/mL)	↓	6 of 6 patients lower Bb ✓
C3 Fragments (ug/mL)	↓	4 of 6 patients lower C3 Fragments[2] ✓
Urinary Ba[1]	↓	6 of 6 patients lower Urinary Ba ✓
Urinary C5b-9[1]	↓	6 of 6 patients lower Urinary C5b-9 ✓
uACR (mg/g)	↓	5 of 6 patients lower uACR[3] ✓

1.Measured by Achillion
2. 2 of 6 remaing patients did not have a readout
3. Patient with higher uACR weighed 86 lb

Two Ongoing Phase 2 Studies

12-Month Open-Label Phase 2 Trial	6-Month Blinded Phase 2 Trial
22 patients enrolled	13 patients enrolled

Key Endpoints

1. Changes in Renal Biopsy Scores
2. Reductions in Proteinuria (urine protein)
3. Changes in eGFR (measure of kidney function)

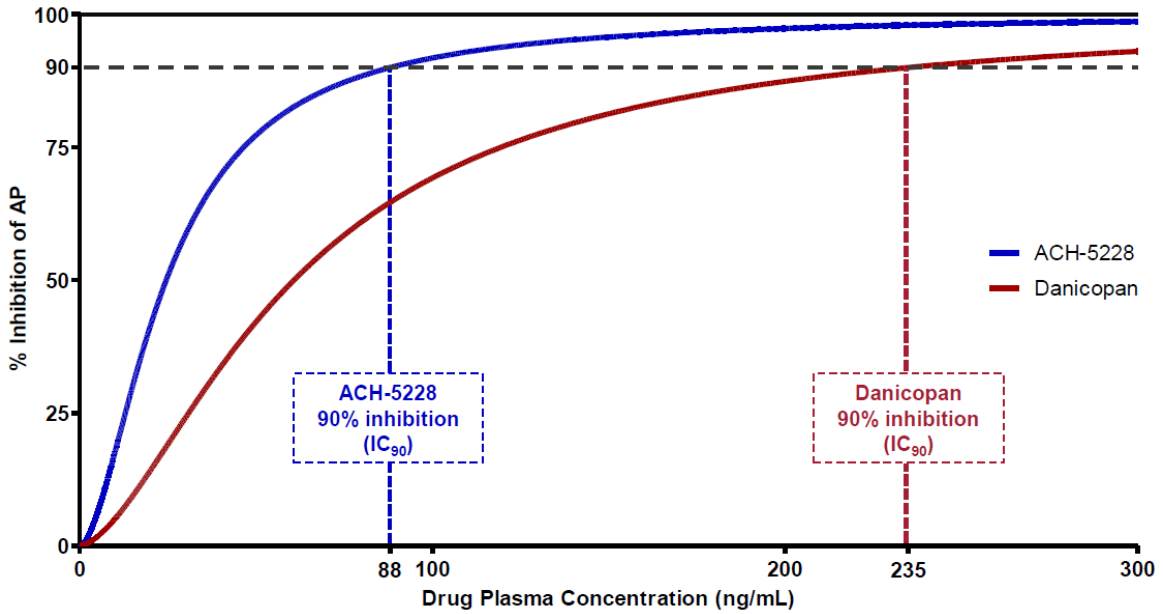
Study completion expected in 2020

Danicopan for C3G has been granted Orphan Drug Designation in the US and the EU

ACH-5228: POTENTIAL BEST-IN-CLASS ORAL COMPLEMENT INHIBITOR

- **Successful Phase 1 multiple ascending dose study**
  - 43 healthy volunteers received oral doses ranging from 40mg to 200mg BID (twice per day) for 14 days[1]
- **When dosed 120mg BID or higher, ACH-5228 achieved near complete and sustained alternative pathway inhibition**
  - AP inhibition of **>95%** at steady state concentrations, as measured by AP Hemolysis and AP Wieslab assays
- **Well tolerated without any severe adverse events over the dose ranges tested**
- **Plan to submit IND Application in Q4 2019**
- **Plan to initiate Phase 2 PNH study in 2020 subject to regulatory feedback**

Multiple Ascending Dose Results



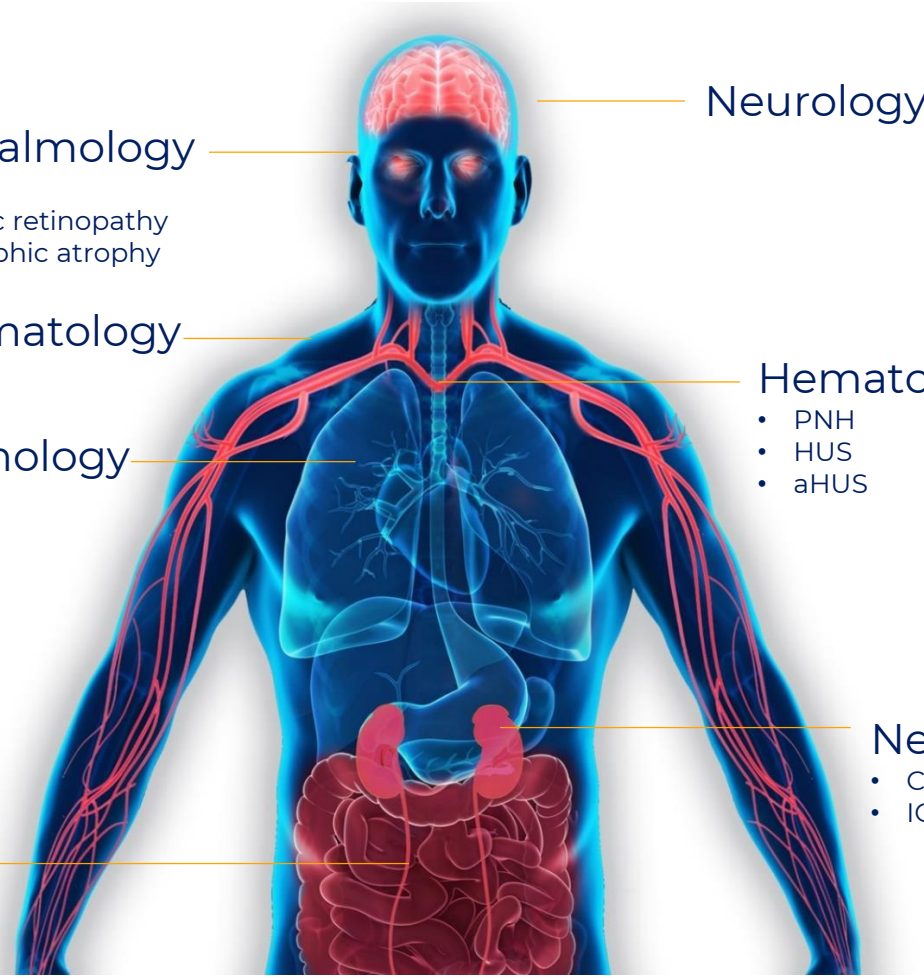
Note: AP = alternative pathway.
>95% AP inhibition at mean steady state trough concentrations in P1 MAD study in healthy volunteers as measured by AP Hemolysis and AP Wieslab assays

1. Study dose included a single dose cohort of 240mg

EXPANDING TO OTHER ALTERNATIVE PATHWAY-MEDIATED RARE DISEASES

- **Established scientific rationale** to treat complement alternative pathway-mediated rare diseases
- Acquisition creates opportunity to leverage Alexion's **leading rare disease development and commercialization capabilities** to accelerate development of portfolio of oral Factor D inhibitors

Multiple Potential Therapeutic Areas of Interest



Neurology

Ophthalmology
- AMD
- Diabetic retinopathy
- Geographic atrophy

Dermatology

Hematology
- PNH
- HUS
- aHUS

Pulmonology

Nephrology
- C3G
- IC-MPGN

Gastroenterology
- Non-alcoholic steatohepatitis (NASH)
- Fatty liver disease
- Cirrhosis

Source: Achillion

Pipeline Update

OPTION TO CO-DEVELOP AND COMMERCIALIZE ELAMIPRETIDE

Primary Mitochondrial Myopathy (PMM)

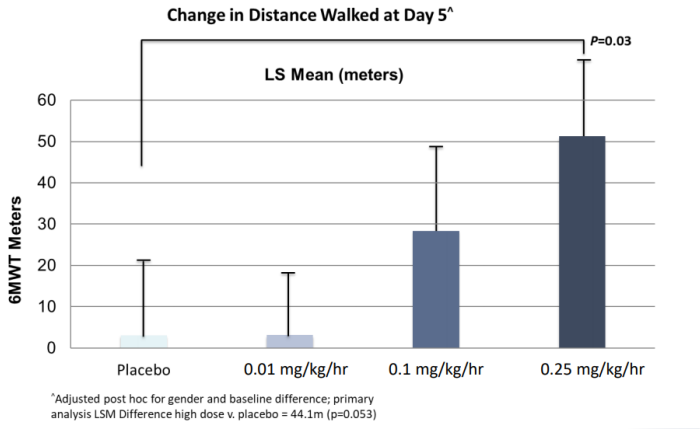
- **Lead indication PMM**: Mitochondrial disorder characterized by debilitating muscle weakness, chronic fatigue, and exercise intolerance. Caused by mitochondrial dysfunction resulting from cardiolipin peroxidation
- No currently approved treatments for PMM. Standard of care is supportive, largely managed by neurologists / neuromuscular specialists

Additional Indications

- **Barth's Syndrome**: Ultra-rare genetic mitochondrial disease with high infant mortality and no approved treatments. Completed Phase 2/3 development
- **Leber's Hereditary Optic Neuropathy (LHON)**: Mitochondrial disease leading to vision loss. Phase 2 trial complete and Phase 3 planning underway

Elamipretide

- Small molecule that binds to and stabilizes cardiolipin, improving mitochondrial function
- Daily subcutaneous auto-injector
- MMPOWER Phase 2 showed improvement in 6 minute-walk test among 'low walker' patients



- MMPOWER Phase 3 Trial underway, top line results expected Q1 2020

ALXN option to co-develop and commercialize elamipretide for mitochondrial diseases

ACHILLION BOLSTERS ALEXION'S EARLY AND ADVANCED CLINICAL PIPELINES

23 Development Programs Planned

Preclinical	Early Clinical	Advanced Clinical	
Internal Complement Programs	ALXN1810 SC (ULTOMIRIS/PH20)	ALXN1840 (WTX101) Wilson Disease	*ULTOMIRIS HSCT-TMA*
Complement Pharma	ABY-039 Rare Autoimmune	ULTOMIRIS QW SC	*CAEL-101 AL Amyloidosis*
Dicerna	ALXN1720 (Anti-C5 Bi-specific)	ULTOMIRIS gMG	*AG10 ATTR Cardiomyopathy*[1]
Zealand	*ULTOMIRIS PPMS*	*ULTOMIRIS NMOSD*	Elamipretide PMM
Next-Gen Treatment for HPP	*ALXN1830 (SYNT001) SC (Healthy Volunteers)*	*ULTOMIRIS ALS*	Elamipretide Barth Syndrome
Next-Gen Factor D Inhibitors	Elamipretide GA in AMD	*ALXN1830 (SYNT001) gMG*	*Elamipretide LHON*
	ACH-5228 PNH	*ALXN1830 (SYNT001) WAIHA*	Danicopan (ACH-4471) PNH with Clinical EVH
	ACH-5228		Danicopan (ACH-4471) C3G

Option to Co-Develop and Commercialize with Stealth Biotherapeutics announced Oct 10th

Subject to Achillion acquisition approval and closure

- Hematology & Nephrology
- Metabolics
- Neurology
- FcRn
- TBD / Other

Italicized = plans to initiate

Note: Caelum Biosciences deal is structured as an option to acquire

[1]Japan only

Closing Remarks

Ludwig Hantson, Ph.D.
Chief Executive Officer

ALIGNED WITH OUR STRATEGY TO DELIVER LONG-TERM GROWTH

Strong Strategic Fit

Leverages Alexion's complement and rare disease development and commercialization expertise

Build and Diversify in Rare Diseases

Broaden Alexion's portfolio with the potential to serve more patients with rare complement-mediated diseases

Disciplined Business Development

Transaction maintains Alexion's financial flexibility to continue to build the pipeline while adding new clinical stage assets

Q&A