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2017 Year-End Earnings and 2018 Update Teleconference

February 22, 2018

Agenda

Glenn Schulman <i>Executive Director, Investor Relations</i>	Introduction	8:30a – 8:35a
Milind Deshpande <i>Chief Executive Officer</i>	Opening Remarks	8:35a – 8:45a
Mary Kay Fenton <i>Executive Vice President and Chief Financial Officer</i>	4 th Quarter and Full Year Financial Results and 2018 Guidance Update	8:45a – 8:50a
Joe Truitt <i>President and Chief Operating Officer</i>	2018 Strategic Objectives and Operating Imperatives	8:50a – 9:00a
Milind Deshpande <i>Chief Executive Officer</i>	Q&A and Concluding Remarks	9:00a – 9:30a



Cautionary Note Regarding Forward-Looking Statements

This presentation includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 that are subject to risks, uncertainties and other important factors that could cause actual results to differ materially from those indicated by such forward-looking statements. Achillion may use words such as “expect”, “anticipate”, “project”, “target”, “intend”, “plan”, “aim”, “believe”, “seek”, “estimate”, “can”, “could”, “focus”, “will”, “look forward”, “continue”, “goal”, “strategy”, “may” and similar expressions to identify such forward-looking statements. These forward-looking statements include statements about Achillion and its business and prospects, including, without limitation, statements regarding drug discovery, research, clinical development, timing of anticipated clinical trials and clinical data for our product candidates, our expectations regarding the potential safety, efficacy and clinical utility of our product candidates, regulatory approval processes, market opportunities, strategic goals, our previous collaboration with Janssen in HCV, intellectual property, competition, and financial results. To the extent that statements contained in this presentation are not descriptions of historical facts, they are forward-looking statements reflecting management’s current beliefs and expectations.

Various important factors may cause differences between our forward-looking statements and actual results, including without limitation, unexpected or unfavorable safety or efficacy data, lower than expected enrollment rates in clinical trials, changes in the competitive landscape for our product candidates, changes in the regulatory environment, changes in market conditions or future demand for our product candidates, the inability to protect our intellectual property, our freedom to operate under third party intellectual property, our need for future capital, the risk of litigation or other disputes, and general market and economic conditions. These and other risks and uncertainties are described in the reports filed by Achillion with the SEC, including its annual report on Form 10-K and quarterly reports on Form 10-Q, and subsequent filings with the SEC from time to time. You should read these reports, including the Risk Factors contained in these reports with the understanding that our actual future results may be materially different from what we currently expect.

All forward-looking statements contained in this presentation speak only as of the date hereof, and Achillion undertakes no obligation to update any of these statements, except as required by law.





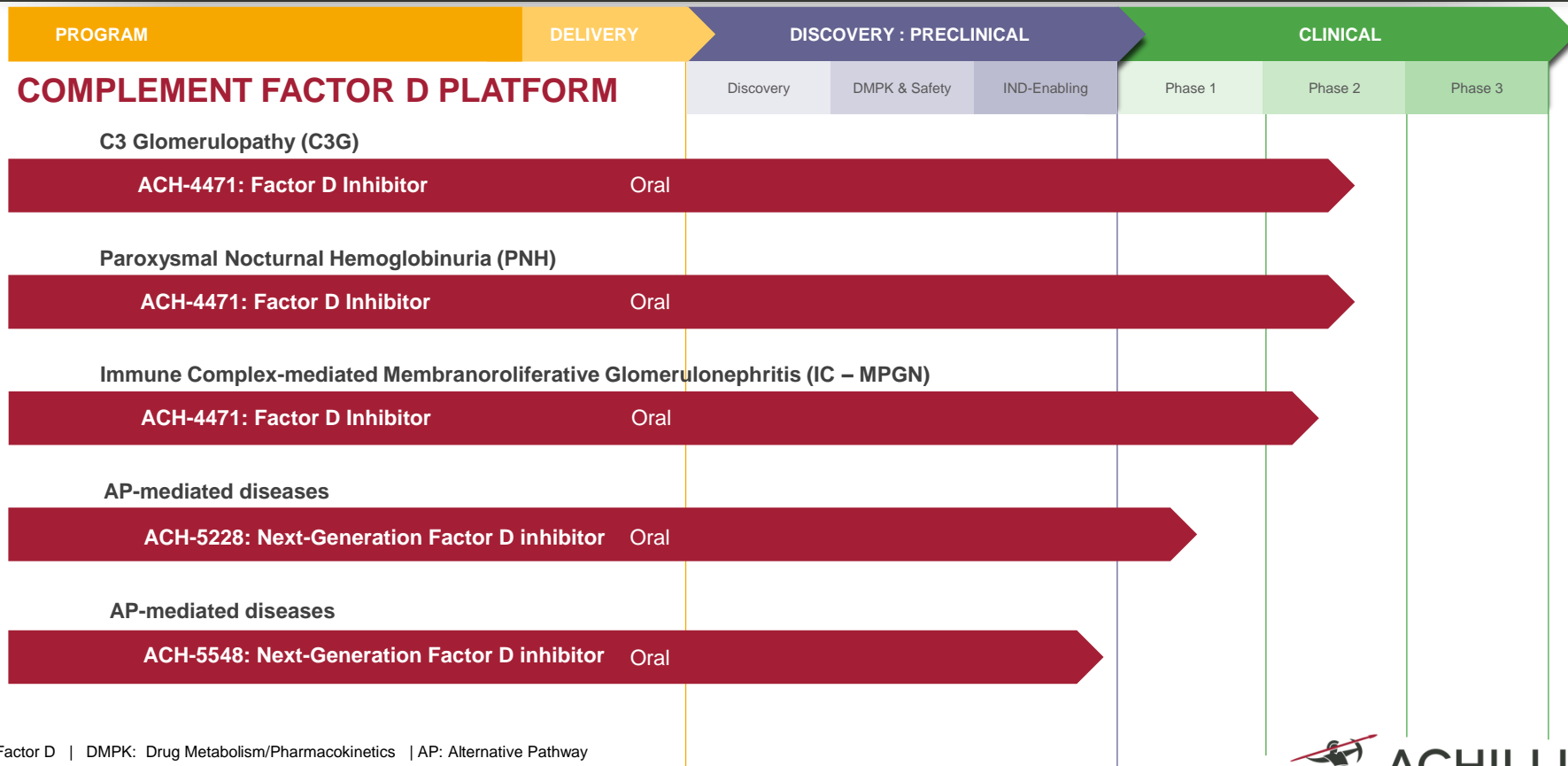
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Milind Deshpande, Ph.D.

Chief Executive Officer

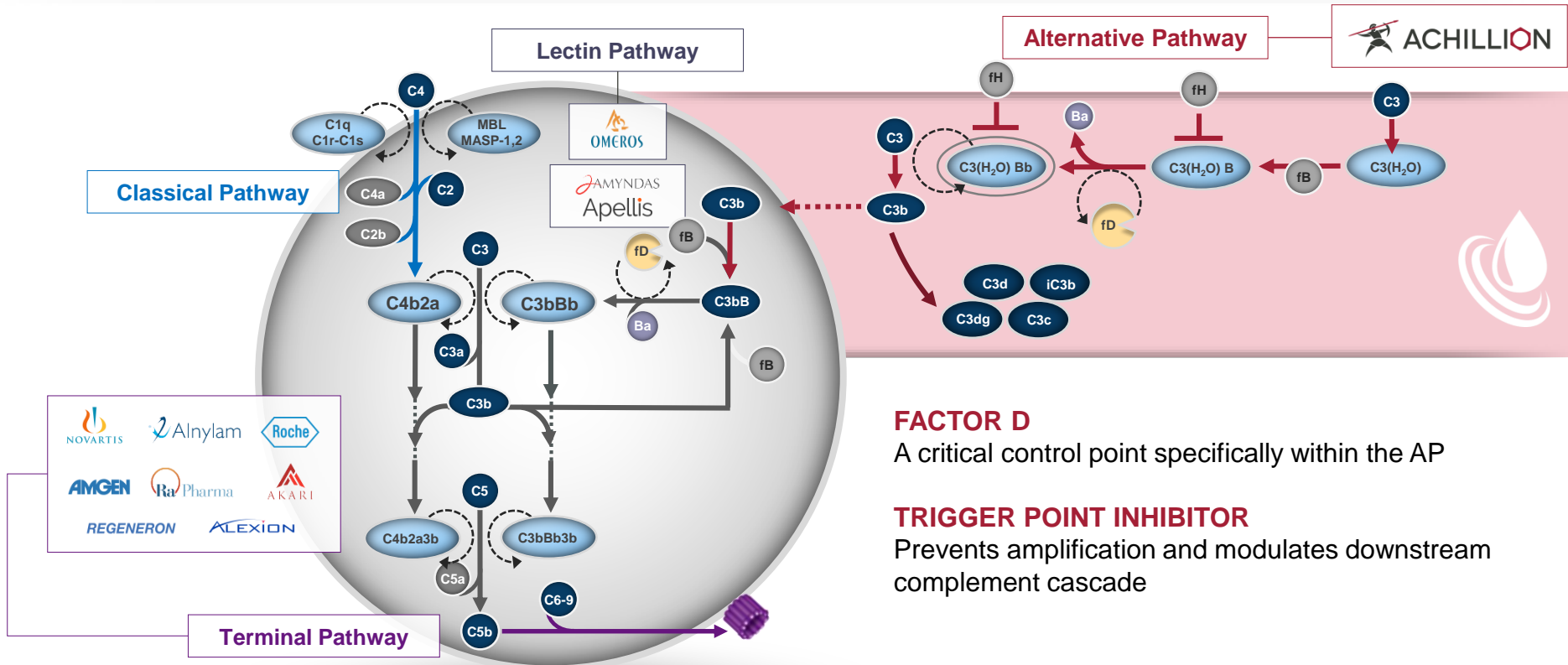
Achillion's Pipeline



fD: Factor D | DMPK: Drug Metabolism/Pharmacokinetics | AP: Alternative Pathway



Mechanism Matters: Factor D Inhibition



FACTOR D

A critical control point specifically within the AP

TRIGGER POINT INHIBITOR

Prevents amplification and modulates downstream complement cascade





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Mary Kay Fenton

Executive Vice President and Chief Financial Officer

Financial Results

	Three Months Ended December 31,		Year Ended December 31,	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Revenue	\$ -	\$ 15,000	\$ -	\$ 15,000
Operating expenses:				
Research and development	15,684	15,029	65,052	59,162
General and administrative	8,671	5,260	24,524	20,703
Total operating expenses	<u>24,355</u>	<u>20,289</u>	<u>89,576</u>	<u>79,865</u>
Loss from operations	<u>(24,355)</u>	<u>(5,289)</u>	<u>(89,576)</u>	<u>(64,865)</u>
Other income (expense):				
Interest income	1,165	874	4,390	3,227
Interest expense	(13)	(14)	(50)	(68)
Net loss	<u>\$ (23,203)</u>	<u>\$ (4,429)</u>	<u>\$ (85,236)</u>	<u>\$ (61,706)</u>
Net loss per share - basic and diluted	<u>\$ (0.17)</u>	<u>\$ (0.03)</u>	<u>\$ (0.62)</u>	<u>\$ (0.45)</u>
Wtd avg shares outstanding - basic and diluted	<u>137,870</u>	<u>136,693</u>	<u>137,180</u>	<u>136,667</u>

YoY R&D expense increased substantially the result of increased clinical expenses with ACH-4471 and increased manufacturing expenses with ACH-5228; YoY G&A expense increased substantially the result of one-time transaction costs in Q4'17



Financial Guidance

	Year Ended December 31,		
	<u>2017 Actual</u>	<u>2018 Estimated Range</u>	
Revenue	\$ -	\$ -	\$ -
Operating expenses:			
Research and development	65,052	58,000 -	60,000
General and administrative	24,524	19,000 -	20,000
Total operating expenses	<u>89,576</u>	<u>77,000 -</u>	<u>80,000</u>
Loss from operations	<u>(89,576)</u>	<u>(77,000) -</u>	<u>(80,000)</u>
Other income (expense):			
Restructuring charge	-	(1,500) -	(1,600)
Interest income	4,390	2,750 -	3,250
Interest expense	(50)	(65) -	(65)
Net loss	<u>\$ (85,236)</u>	<u>\$ (75,815) -</u>	<u>\$ (78,415)</u>
Net loss per share - basic and diluted	<u>\$ (0.62)</u>	<u>\$ (0.55) -</u>	<u>\$ (0.58)</u>
Wtd avg shares outstanding - basic and diluted	<u>137,180</u>	<u>137,894 -</u>	<u>138,144</u>

With more than \$330 million in cash and cash equivalents, Achillion has the resources to create shareholder value in 2018 and well beyond





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Joseph Truitt

President and Chief Operating Officer

Achillion's Value Proposition

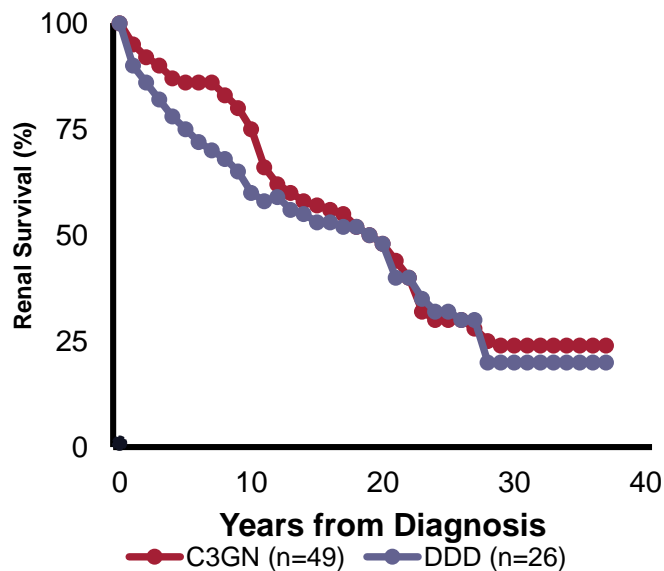
- ◆ **Established proof-of-concept with ACH-4471 for the treatment of C3G**
 - Significant improvement in proteinuria demonstrating preliminary proof-of-concept (PoC); coupled with significant improvement in complement biomarkers demonstrating target engagement
 - Potential disease-modifying therapy for highly motivated patient population where no current treatment available
- ◆ **Established proof-of-concept with ACH-4471 for the treatment of PNH**
 - Good tolerability observed at 200mg TID dose after more than six months of treatment
 - Demonstrated both clinical (hemoglobin) and biomarker (C3 fragment deposition on PNH erythrocytes) improvements for balanced ability to address both EVH and IVH in patients
- ◆ **Factor D platform represents an oral, potentially disease-modifying therapy in multiple diseases and therefore has strong potential alliance opportunities**



A Rare Disease with No FDA-Approved Treatment

- ◆ C3G includes both **Dense Deposit Disease (DDD)** and **C3 glomerulonephritis (C3GN)**
 - Approximately 4,000 U.S. patients, more than 4,000 in Europe, and greater than 1,000 in Japan
- ◆ There are **no approved treatments** indicated for patients with C3G
 - Non-specific treatment approaches include blood pressure control and broad immunosuppression
- ◆ Significant unmet medical need as nearly half of **C3G patients progress to end-stage renal disease**
 - 30-50% progress to ESRD within 10 years
 - Significant disease burden at onset – inflammation, profound fatigue and weakness
 - Greater than 50% of patients experience disease recurrence post renal transplant, with a 50% chance of graft loss

DDD AND C3GN
IMPACT ON RENAL SURVIVAL



Barbour et al. (2015); NICE C3G Evidence Summary (2015);

2018 Goals and Milestones

Near-term Clinical Development Plans

Compound	Indication	Anticipated Next steps
ACH-4471	C3G & IC-MPGN	<ul style="list-style-type: none">• 14-day Ph 2 Group 2 interim data 3Q18• 12-month open label Ph 2: Interim data 4Q18• 6-month randomized double-blind C3G Ph 2: Data available in 2019
	PNH	<ul style="list-style-type: none">• Monotherapy Ph 2: Ongoing and interim data 4Q18• Add-on trial Ph 2: Interim data 4Q18
	XR Program	<ul style="list-style-type: none">• Bioavailability study interim data 1H18
Next-Gen Compounds	ACH-5228	<ul style="list-style-type: none">• Ph 1 interim data in 2H18
	ACH-5548	<ul style="list-style-type: none">• Initiate Ph 1 in 2H18



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Thank you for your participation. Follow-up questions can be directed to Glenn Schulman at gschulman@achillion.com.

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