Soliris® in NMOSD
Phase 3 PREVENT Study Topline Results

September 24, 2018

Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve risks and uncertainties relating to future events and the future performance of Alexion, including statements related to: the prospect of Alexion bringing forward an effective treatment for NMOSD patients who currently have no approved therapies; the potential of Soliris® to transform NMOSD treatment given the significant unmet need with no currently approved treatments; Soliris® will be an effective treatment for patients with NMOSD and could mark a turning point in the treatment of the disease; the PREVENT study results are the basis for hope for NMOSD patients, families and the neurology community; the PREVENT study (and Soliris®) represent a potential significant step forward in the treatment of NMOSD; the beneficial impact that the relapse reduction could have for patients with NMOSD using Soliris®; Soliris® (and its ability to inhibit complement) can have a profound effect on NMOSD and its potential to be a life-changing treatment for NMOSD patients; Soliris® has the potential to be the first approved treatment for NMOSD; future plans to engage with regulatory authorities (and the timing thereof) and future planned submission of regulatory applications for review and approval by regulatory authorities in certain countries for Soliris® as a treatment for NMOSD; future plans for the extension of the clinical trial (and make supplemental regulatory filings with information from such extension trial); future plans for commercial launch of Soliris® as a treatment for NMOSD; and the potential medical benefits of Soliris® for the treatment of NMOSD and other diseases. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those expected by these forward looking statements, including for example: the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations (including Soliris® as a treatment for NMOSD); the inability to timely provide (or failure to provide) the product safety and efficacy information required by regulatory authorities for Soliris® as a treatment for NMOSD; the inability to submit regulatory applications for Soliris® as a treatment for NMOSD for review and approval by certain governmental authorities (or an unexpected delay in the timeframes for such submissions) due to increased expense, manufacturing delays or other reasons; our products not gaining acceptance among patients (and providers or third party payers) for certain indications (due to cost or otherwise), the inability to develop future clinical study programs for certain product delivery mechanisms (or the failure of those programs to meet safety and efficacy goals); unforeseen safety issues resulting from the administration of products and product candidates in patients, including serious complications or side effects; the inability to timely and cost-effectively develop programs for existing products for new indications (or the failure to obtain regulatory approval for use in such new indications); decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products (or the indications of such products); delays, interruptions, or failures in the manufacture and supply of our products and our product candidates; the introduction of competing drugs and product candidates for NMOSD and other indications; failure to satisfactorily address matters raised by the FDA and other regulatory agencies; the possibility that current rates of adoption of our products are not sustained (or do not meet expected future rates); the possibility that clinical trials of our product candidates could be delayed; the adequacy of our pharmacovigilance and drug safety reporting processes; the risk that third party payers (including governmental agencies) will not reimburse or continue to reimburse for the use of our products (or proposed future products) at acceptable rates or at all; risks that our future business development transactions and internal development efforts do not result in products that obtain regulatory approval, are not accepted by the market or otherwise do not expand our product portfolio; 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 other anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with the diseases that our products treat are inaccurate, 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 Alexion's 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.

Provided September 24, 2018, 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.

Agenda

Introduction	Susan Altschuller, Ph.D., Investor Relations
Opening Remarks	Ludwig Hantson, Ph.D., Chief Executive Officer
Phase 3 NMOSD Results	John Orloff, M.D., Head of R&D
Closing Remarks	Ludwig Hantson, Ph.D., Chief Executive Officer
Q&A	Brian Goff, Chief Commercial Officer, & Paul Clancy, Chief Financial Officer, available for Q&A

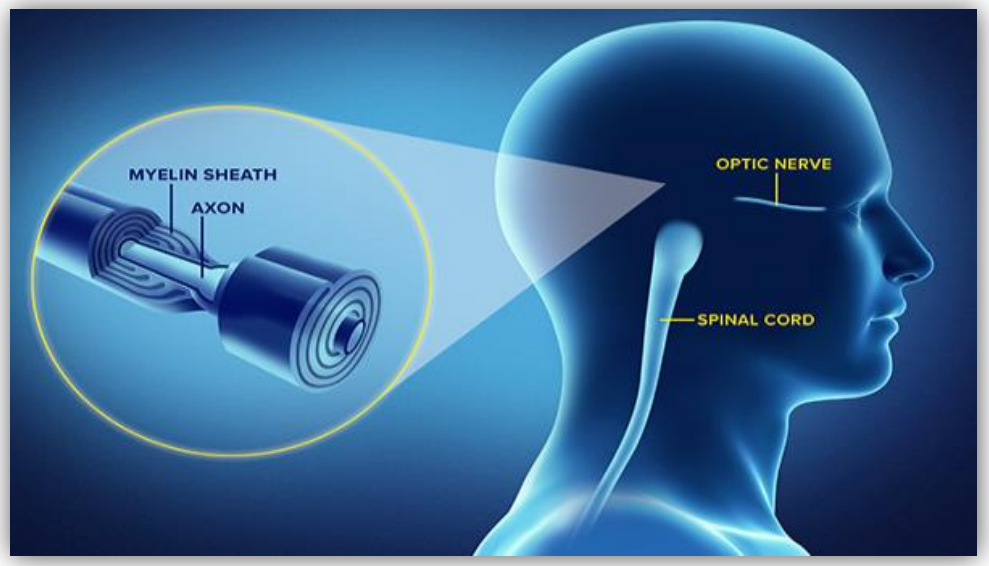
CEO Opening Remarks

Ludwig Hantson, Ph.D.
Chief Executive Officer

Successful Phase 3 Study of Soliris® in NMOSD, A Rare Neurologic Disease with No Approved Therapies

In the Phase 3 PREVENT study in patients with Neuromyelitis Optica Spectrum Disorder (NMOSD):

- Soliris® is superior to placebo with a relapse risk reduction of 94.2%, HR 0.058 ($p < 0.0001$) based on primary endpoint of time to first adjudicated on-trial relapse
- 97.9% of Soliris® patients relapse free at 48 weeks vs. 63.2% for placebo
- Well tolerated with a safety profile consistent with Soliris® in previous clinical studies and real-world use in all three approved indications
- Opportunity to transform the treatment of NMOSD given significant unmet need with no currently approved treatments



NMOSD: A Disabling Disease of Inflammation and Demyelination Of The Optic Nerve and Spinal Cord

Description

- Rare, chronic, potentially life-threatening neurologic disease that attacks the optic nerve and spinal cord

Etiology

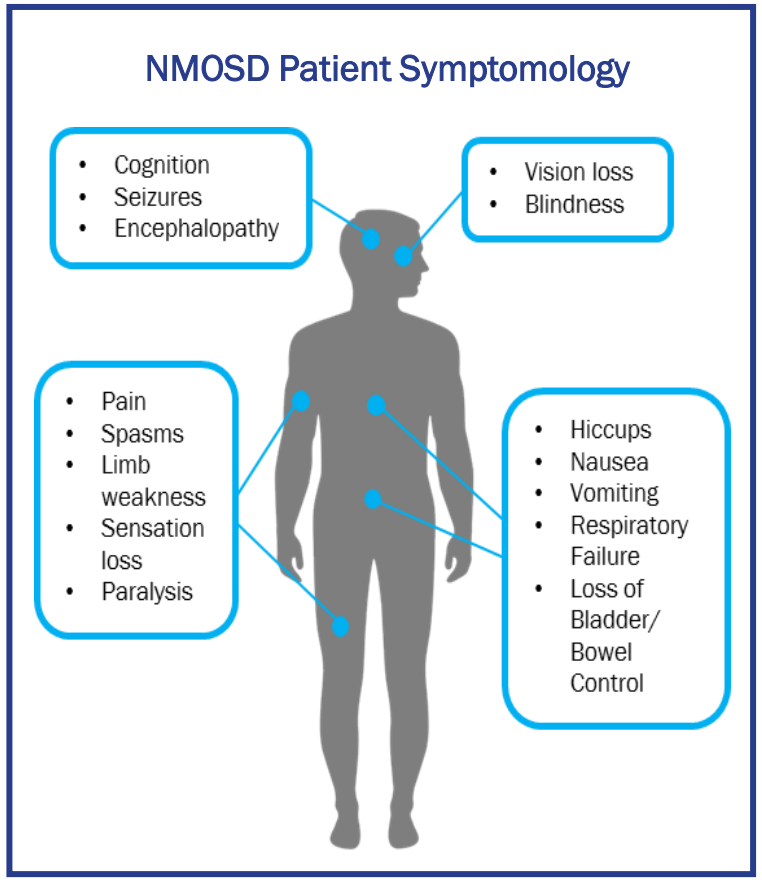
- Patients typically present with Optic Neuritis (ON) and/or Transverse Myelitis (TM) symptoms, initial treatment course depends on severity at presentation
- Worsening disability with each successive relapse

Epidemiology

- Estimated prevalence is ~8K in the US, ~8K in EU5
  - ~75% patients anti-AQP4+
- Time to diagnosis depends largely on initial presentation, physician awareness
- Predominantly affects women

Unmet need

- No approved therapies for the treatment of NMOSD
- Current treatment includes corticosteroids and other immunosuppressants (e.g. rituximab)
- Relapse prevention is the primary treatment goal



Source: UpToDate; MayoClinic

NMOSD Diagnosis Can Be Delayed, Treatment Options Are Limited & Patients Have Significant Disability

Patient Journey

Patients typically present with either Optic Neuritis (ON) or Transverse Myelitis (TM) symptoms

Presentation to physician depends on attack symptomology

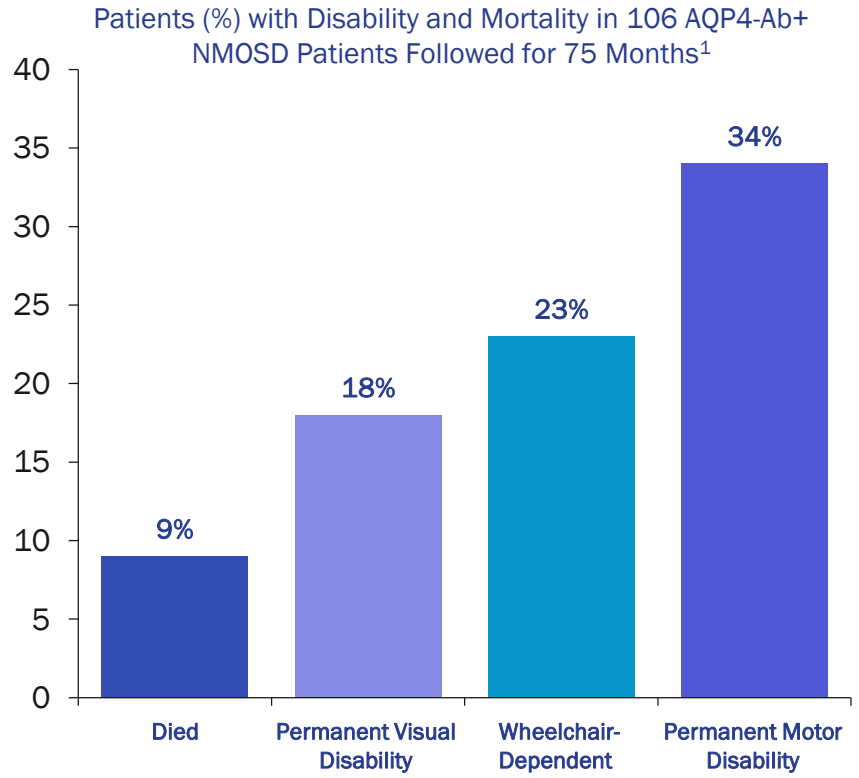
- Emergency Room Physician
- Primary Care Physician
- Ophthalmologist
- Other (rheumatologist, neurologist, etc.)

Time to diagnosis depends on initial presentation and physician awareness

ON patients | TM patients | ON/TM patients

Ongoing management by neurologists; No approved therapies and limited options for relapsing patients

Patients at Risk of Disability or Death



Note: Pre-2000, NMOSD had 33% mortality at 5 years due to C-spinal cord damage

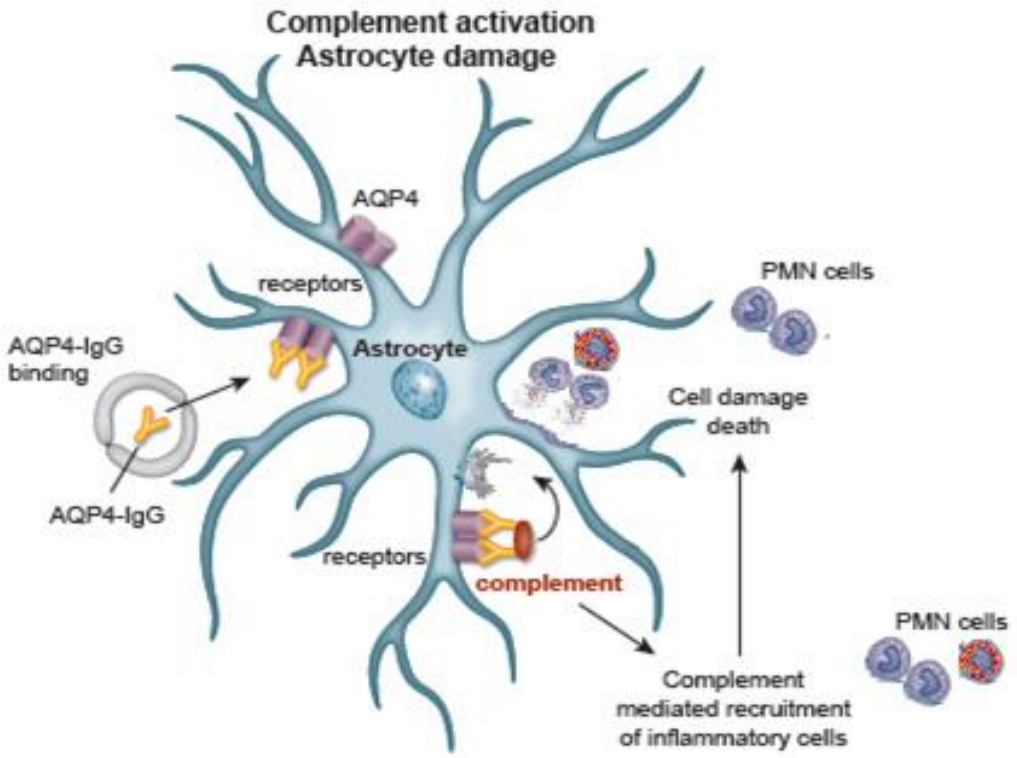
1. *Kitley, J., et al. Brain. 2012; 135: 1834-1849.*

Soliris® NMOSD
Phase 3 PREVENT Study Results

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

The Role of Complement in NMOSD

Complement Activation in NMOSD

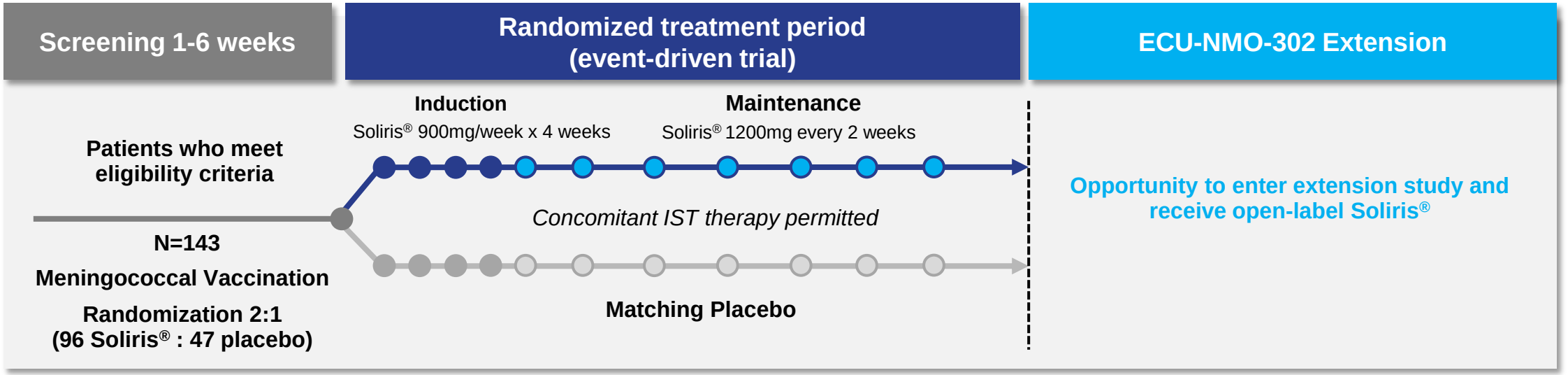


- AQP4-specific antibody (IgG) binds to AQP4 astrocyte protein triggering complement activation
- Immune activation through complement leads to astrocyte and oligodendrocyte injury, causing demyelination
- Pro-inflammatory effects can be triggered by complement factor C5a, leading to the activation and infiltration of eosinophils, neutrophils and granulocytes in the spinal cord
- Complement activation, deposition of the MAC, and destruction of astrocytes by lysis cause necrosis, neuronal injury, and demyelination

1. *Papadopoulos, M.C., et al. Nature Rev Neurol. 2014;10:493-506.*
2. *Verkman, A.S., Annu Rev Med. 2012;63:303–316.*

Phase 3 PREVENT Clinical Program Design

- Patients with relapsing neuromyelitis optica spectrum disorders (NMOSD) who are anti-AQP4 antibody positive
- ECU-NMO-301: Pivotal Phase 3 randomized, double-blind, placebo-controlled, multi-center, event-driven trial to evaluate the efficacy and safety of Soliris® in patients with relapsing NMOSD
  - Primary Endpoint: Time to first adjudicated on-trial relapse
  - Secondary Endpoints: Adjudicated on-trial annualized relapse rate and measures of disability and quality of life



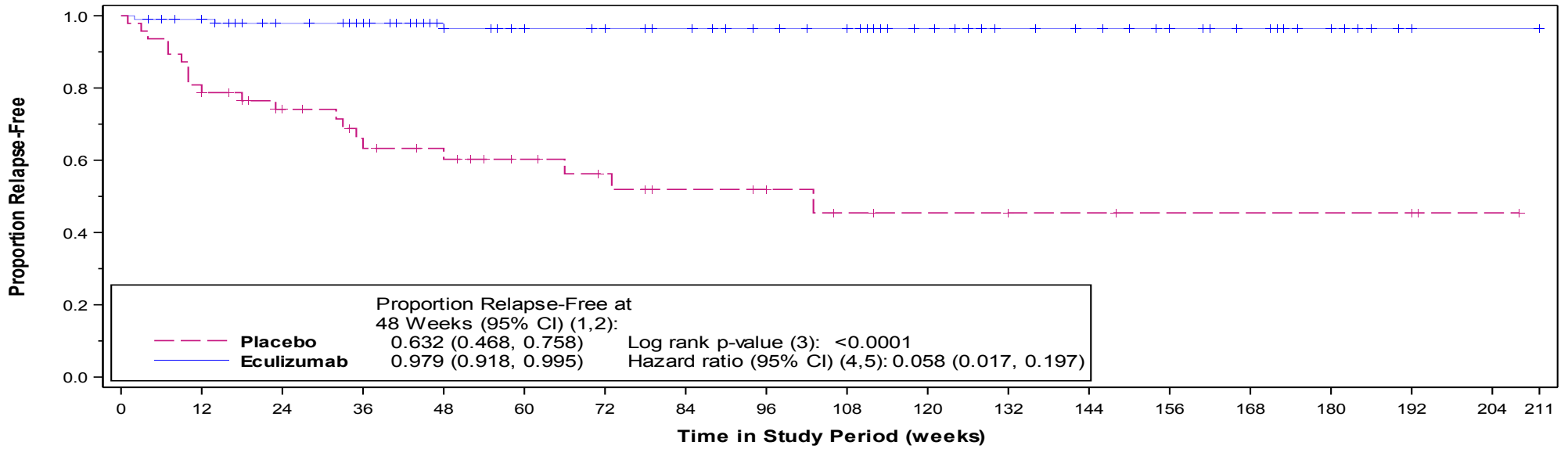
Phase 3 PREVENT Study: Patient Baseline Characteristics

Variable	Statistic	Placebo (n= 47)	Soliris® (n=96)	Total (n=143)
Age at First Dose (Years)	Mean (SD) Median	45.0 (13.29) 44.0	43.9 (13.32) 45.0	44.3 (13.27) 45.0
Sex				
Males	n (%)	5 (10.6)	8 (8.3)	13 (9.1)
Females	n (%)	42 (89.4)	88 (91.7)	130 (90.9)
Race				
Asian	n (%)	15 (31.9)	37 (38.5)	52 (36.4)
Japanese Patients	n (%)	5 (10.6)	9 (9.4)	14 (9.8)
Black or AA	n (%)	8 (17.0)	9 (9.4)	17 (11.9)
White	n (%)	24 (51.1)	46 (47.9)	70 (49.0)
Other	n (%)	0 (0)	4 (4.2)	4 (2.8)
Region				
Americas	n (%)	15 (31.9)	29 (30.2)	44 (30.8)
Europe	n (%)	19 (40.4)	32 (33.3)	51 (35.7)
Asia-Pacific	n (%)	13 (27.7)	35 (36.5)	48 (33.6)
Historical Annualized Relapse Rate within 24 months prior to Screening	Mean (SD) Median	2.07 (1.037) 1.92	1.94 (0.896) 1.85	1.99 (0.943) 1.92
No IST Usage	n (%)	13 (27.7)	21 (21.9)	34 (23.8)
Steroids Alone	n (%)	11 (23.4)	16 (16.7)	27 (18.9)

Primary Efficacy Results: Time to First Adjudicated On-trial Relapse

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

Kaplan-Meier Survival Estimates for Time to First Adjudicated On-Trial Relapse



Note: Patients who did not experience an adjudicated on-trial relapse were censored at the end of the study period.
Stratified analyses are based on four randomization strata: (i) low EDSS at randomization (<= 2.0), (ii) high EDSS (>=2.5 to <= 7) and treatment naïve at randomization, (iii) high EDSS (>=2.5 to <= 7) and continuing on the same IST(s) since last relapse at randomization, (iv) high EDSS (>=2.5 to <= 7) and changes in IST(s) since last relapse at randomization.
(1) Based on the Kaplan-Meier product limit method, (2) Based on the complementary log-log transformation, (3) Based on a stratified log-rank test,
(4) Based on a stratified Cox proportional hazards model, (5) Wald confidence interval

Strong Efficacy for Adjudicated On-Trial and On-Trial Relapse

Variable / Statistic	Adjudicated On-trial Relapse		On-trial Relapse	
Treatment Group	Placebo (n = 47)	Soliris® (n = 96)	Placebo (n = 47)	Soliris® (n = 96)
Patients with a Relapse (%)	20 (42.6)	3 (3.1)	29 (61.7)	14 (14.6)
Patients Relapse Free at 48 Weeks	63.2%	97.9%	50.6%	89.3%
Hazard Ratio (95% CI)		0.058 (0.017, 0.197)		0.180 (0.095, 0.343)
Risk Reduction (%)		94.2% (p<0.0001)		82.0% (p<0.0001)
Annualized Relapse Rate Reduction (%)		95.5%		85.3%

Phase 3 PREVENT Study: Safety Summary

	Placebo (n = 47) Pt-years (PY) = 53.1			Soliris® (n = 96) Pt-years (PY) = 172.8		
	Events n	Rate per 100 PY	Patients n (%)	Events n	Rate per 100 PY	Patients n (%)
Any Adverse Event (AE)	617	1,160.9	45 (95.7)	1,295	749.3	88 (91.7)
Any Serious AE (SAE)	47	88.4	26 (55.3)	53	30.7	30 (31.3)
Death			0 (0.0)			1 (1.0)
SAEs Leading to Withdrawal of Study Drug	2	3.8	2 (4.3)	0	0.0	0 (0.0)
Meningococcal Infections	0	0	0 (0.0)	0	0.0	0 (0.0)
Most Common AEs (>15%)						
Upper respiratory tract infection	10	18.8	6 (12.8)	54	31.2	28 (29.2)
Headache	20	37.6	11 (23.4)	95	55.0	22 (22.9)
Nasopharyngitis	15	28.2	9 (19.1)	50	28.9	20 (20.8)
Nausea	19	35.7	12 (25.5)	30	17.4	16 (16.7)
Urinary tract infection	13	24.5	10 (21.3)	45	26.0	13 (13.5)
Diarrhea	19	35.7	7 (14.9)	23	13.3	15 (15.6)

Next Steps Towards Approval for Soliris® in NMOSD

- Successful Phase 3 PREVENT NMOSD trial
  - Achieved statistical significance (P<0.0001) on primary endpoint of time to first adjudicated on-trial relapse; Hazard Ratio (HR) of 0.058 representative of relapse risk reduction of 94.2%
  - Generally well tolerated with safety profile consistent with previous clinical trials and real-world use of Soliris®
  - Potential to be the first approved treatment for NMOSD, addressing significant unmet need
- Plan to engage with regulatory authorities in the coming months to discuss Phase 3 results and potential regulatory filing
- Ongoing extension trial to supplement filing with long-term safety and efficacy data
- Initial launch planning will leverage footprint and learnings from Soliris® launch in gMG

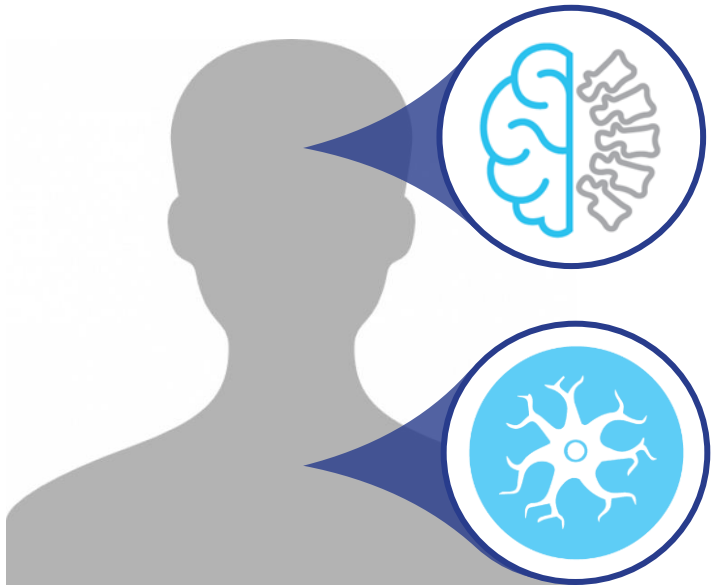
Closing Remarks

Ludwig Hantson, Ph.D.

Chief Executive Officer

Phase 3 PREVENT Trial Presents Opportunity to Expand Rare Neurology Franchise

Complement plays key role in numerous rare neurology indications



Hematology	Metabolic	Neurology
PNH	HPP	gMG
aHUS	LAL-D	NMOSD
	Wilson Disease	

Q&A