ALXN1210 PNH Naïve Phase 3 Study Results

March 15, 2018

Forward-Looking Statements

This presentation contains forward-looking statements, including statements related to Alexion's development plans for ALXN1210, the potential medical benefits of ALXN1210 for the treatment of PNH, and Alexion's future clinical, regulatory and commercial plans for ALXN1210. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products, delays, 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, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations, the possibility that current rates of adoption of Soliris in PNH, aHUS or other diseases are not sustained, 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 payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, the possibility that expected tax benefits will not be realized, assessment of impact of recent accounting pronouncements, potential declines in sovereign credit ratings or sovereign defaults in countries where we sell our products, 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 anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with the diseases that our products treat are inaccurate, the risks of changing foreign exchange rates, risks relating to the potential effects of the Company's restructuring and relocation of its corporate headquarters, 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 Annual Report on Form 10-K for the period ended December 31, 2017 and in our other filings with the SEC. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

Opening Remarks

Ludwig Hantson, Ph.D.
Chief Executive Officer

ALXN1210: Successful Pivotal Phase 3 Study

ALXN1210 met the high hurdle set by a rigorous study design in complement inhibitor treatment-naïve PNH patients:

- ☑ Achieved non-inferiority to Soliris® on co-primary endpoints of transfusion avoidance and LDH normalization
- ☑ Achieved non-inferiority on all 4 key secondary endpoints
- ☑ Numeric results in favor of ALXN1210 on all co-primary and key secondary endpoints
- ☑ Was well tolerated with a safety profile consistent with Soliris®
- ☑ Achieved immediate, complete and sustained C5 inhibition with every 8 week dosing interval vs. every 2 weeks with Soliris®

Co-Primary Endpoints Met Non-Inferiority

Transfusion Avoidance (TA)

ALXN1210 achieved non-inferiority vs. Soliris® on TA

- Transfusion Avoidance:
  73.6% for ALXN1210, **66.1%** for Soliris®
- Difference in proportion of patients of **6.8%** (95% CI: -4.7, 18.1)

LDH Normalization

ALXN1210 resulted in rapid and sustained normalization of LDH levels achieving non-inferiority vs. Soliris®

- LDH Normalization[1]:
  53.6% for ALXN1210, **49.4%** for Soliris®
- Odds Ratio of **1.19** (95% CI: 0.8, 1.77)

Note: ALXN1210 demonstrated non-inferiority to Soliris® for the two co-primary endpoints since the lower (LB) or upper bounds (UB) of the 95% confidence intervals (CI) for the treatment effect estimates were greater or smaller than the respective pre-defined non-inferiority thresholds.
[1]Normalization of LDH levels was evaluated every 2 weeks from Day 29 through Week 26 and calculated using a repeated measure model
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Achieved Non-Inferiority on All 4 Key Secondary Endpoints

LDH Reduction	**ALXN1210 resulted in rapid and sustained reductions in LDH**	▪ **-76.8%** for ALXN1210, **-76.0%** for Soliris® ▪ Δ of **-0.83%** (95% CI: -5.2%,3.6%)
FACIT-Fatigue Scale	**ALXN1210 resulted in clinically meaningful QoL improvements as measured by the FACIT-Fatigue Scale**	▪ **7.1** for ALXN1210, **6.4** for Soliris® ▪ Δ of **0.67** (95% CI: -1.2, 2.6)
Breakthrough Hemolysis	**ALXN1210 resulted in a low proportion of patients with breakthrough hemolysis**	▪ **4.0%** for ALXN1210, **10.7%** for Soliris® ▪ Δ of **-6.7%** (95% CI: -14.2%,0.18%)
Stabilization of Hemoglobin	**ALXN1210 resulted in a high proportion of patients achieving stabilization of hemoglobin**	▪ **68.0%** for ALXN1210, **64.5%** for Soliris® ▪ Δ of **2.9%** (95% CI: -8.8%,14.6%)

Note: ALXN1210 demonstrated non-inferiority to Soliris® for the two co-primary endpoints since the lower (LB) or upper bounds (UB) of the 95% confidence intervals (CI) for the treatment effect estimates were greater or smaller than the respective pre-defined non-inferiority thresholds.

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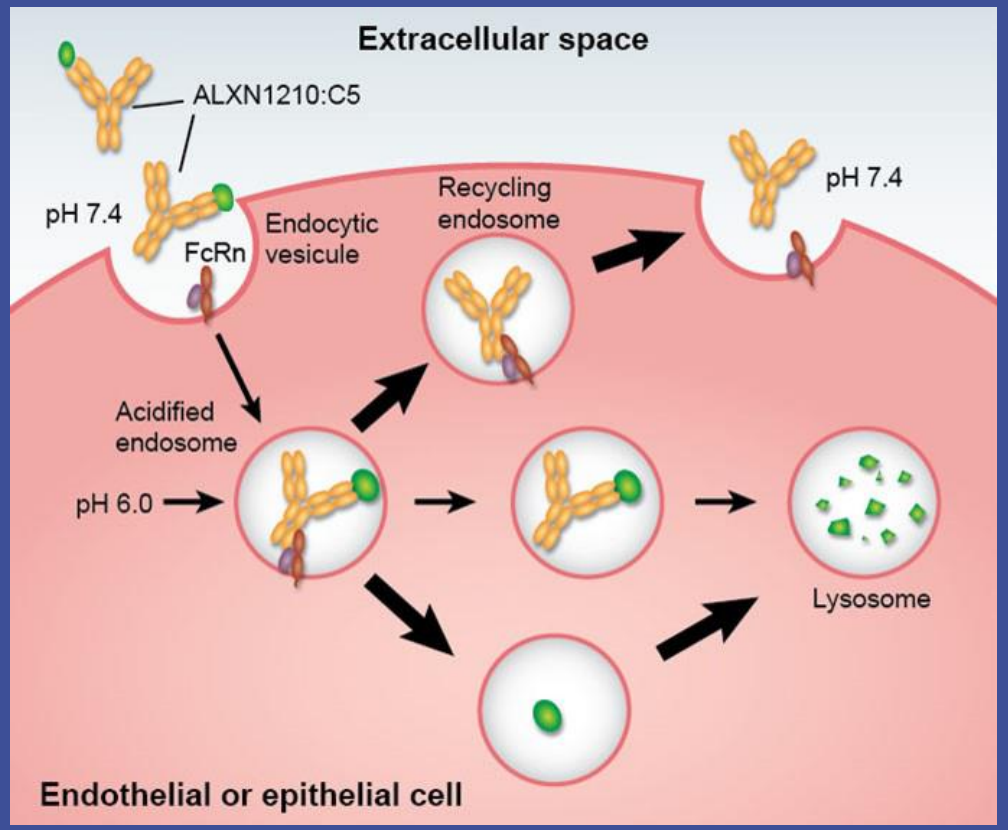
ALXN1210 PNH Naïve Phase 3 Study Results

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

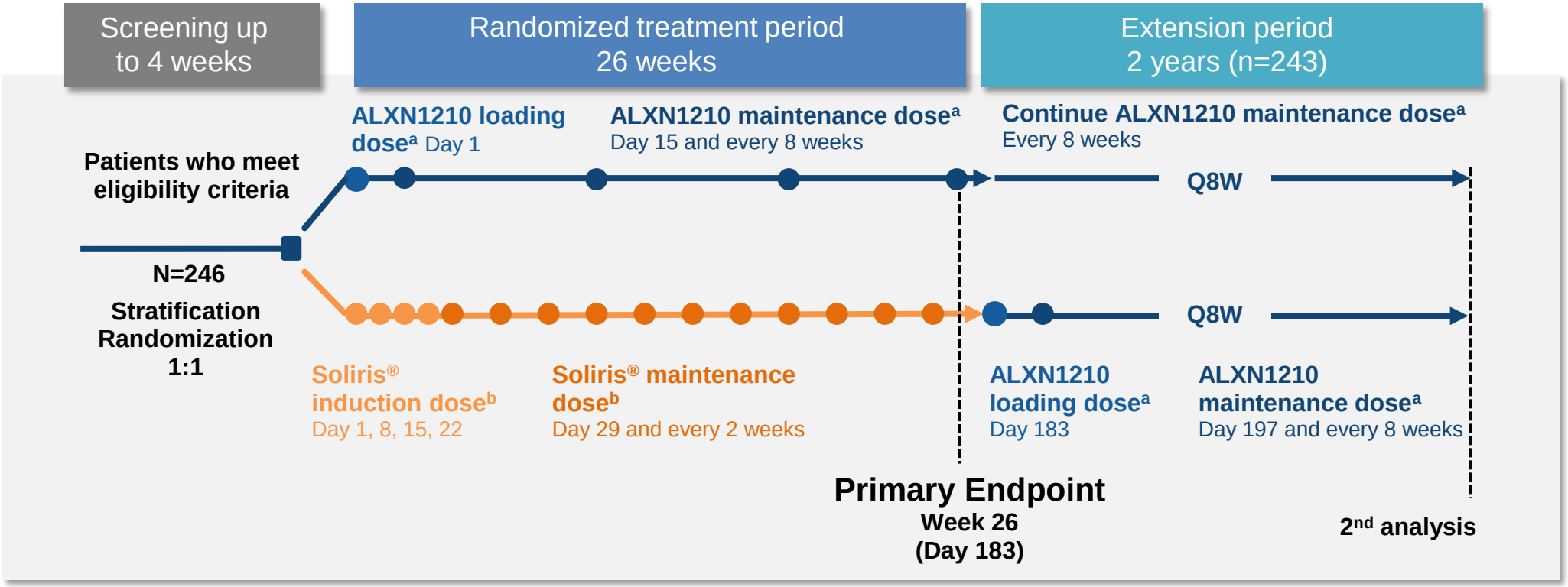
ALXN1210: Highly Innovative Next-Generation C5 Inhibitor

ALXN1210 Innovative Engineering



- Immediate, complete, sustained reduction of free C5 activity ≥99%
  - Engineered for potent inhibition and enhanced Fc receptor recycling
  - Terminal half-life of 42 days
- Orphan Drug Designation in PNH in the US and EU; aHUS in US
- Composition of matter patent through 2035 in ~50 countries

Phase 3 Trial Design in PNH-Naïve Patients



- **Primary Objective**: Assess non-inferiority of ALXN1210 compared to Soliris® in complement inhibitor treatment-naive patients with PNH
- **Co-Primary Endpoints**: **Transfusion Avoidance** and **Normalization of LDH**

[a]ALXN1210 dosage: loading dose=2400 mg for patients weighing ≥ 40 to < 60 kg, 2700 mg for patients weighing ≥ 60 to < 100 kg, 3000 mg for patients weighing ≥ 100 kg; maintenance dose=3000 mg for patients weighing ≥ 40 to < 60 kg, 3300 mg for patients weighing ≥ 60 to < 100 kg, 3600 mg for patients weighing ≥ 100 kg. [b]Soliris® dosage: induction dose=600 mg; maintenance dose=900 mg.
NCT02946463. Clinical Trial.gov website. https://clinicaltrials.gov/ct2/show/NCT02946463.
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Well-Balanced Baseline Characteristics

		Statistic	ALXN1210 (N=125)	Soliris® (N=121)
Sex	Males	N (%)	65 (52%)	69 (57%)
	Females		60 (48%)	52 (43%)
Age at Infusion (yrs)		Mean (SD)	44.8 (15.2)	46.2 (16.2)
Ethnicity (Not Hisp./Lat.)		N (%)	116 (93%)	102 (84%)
Race	Asian	N (%)	72 (58%)	57 (47%)
	Japan		*19 (15%)*	*15 (12%)*
	Caucasian		43 (34%)	51 (42%)
	Other		10 (8%)	13 (11%)
Body Weight (kg)		Mean (SD)	68.2 (15.6)	69.2 (14.9)
LDH (U/L) at Baseline		Mean (SD)	1,634 (778.8)	1,578 (727.1)
LDH (Randomization Strata)	1.5 - <3xULN	N (%)	18 (14%)	16 (13%)
	> 3xULN		107 (86%)	105 (87%)
pRBC (Randomization Strata)	0 Units	N (%)	23 (18%)	21 (17%)
	1-14 Units		79 (63%)	78 (64%)
	>14 Units		23 (18%)	22 (18%)
Age at PNH Diagnosis		Mean (SD)	37.9 (14.9)	39.6 (16.7)
Yrs PNH Diagnosis to Consent		Mean (SD)	6.7 (8.1)	6.4 (7.5)
PNH Clone Size	RBC Type II	Mean (SD)	12.4 (20.5)	13.7 (17.7)
	RBC Type III		26.3 (17.2)	25.2 (16.9)
	Total RBC		38.4 (23.7)	38.7 (23.2)
	Granulocytes		84.2 (21.0)	85.3 (19.0)
	Monocytes		86.9 (18.1)	89.2 (15.2)

Review of Efficacy Endpoint Measures

Co-Primary Endpoints

1. **Transfusion Avoidance**
   - Defined as the proportion of patients who remain transfusion free and do not require a transfusion per protocol specified guidelines through Day 183

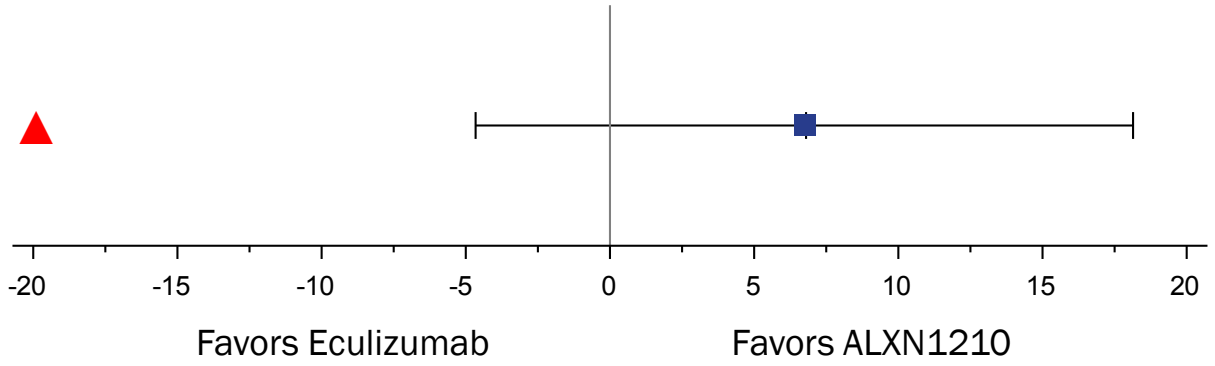
2. **LDH Normalization**
   - Defined as LDH ≤ 1x ULN evaluated every 2 weeks from Day 29 through Day 183

Key Secondary Endpoints

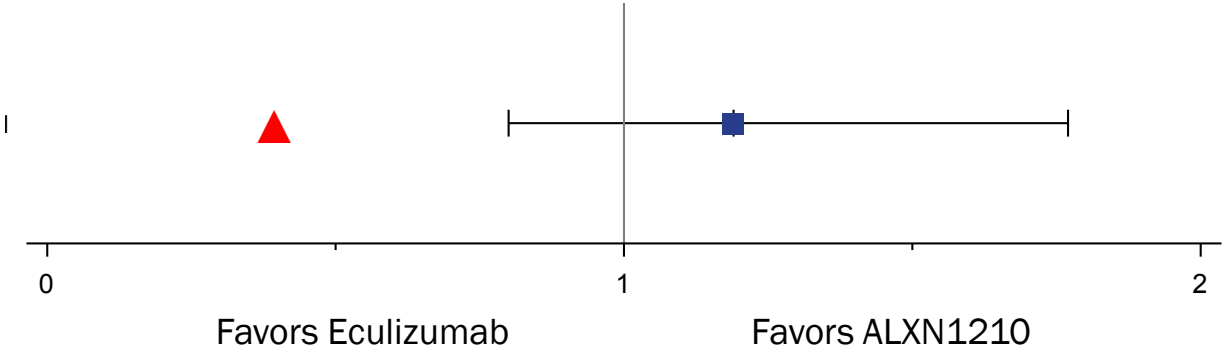
1. **Percentage Change in LDH from Baseline (BL) to Day 183**
2. **Change in HRQoL as Assessed by FACIT-Fatigue from BL to Day 183**
3. **Proportion of Patients with Breakthrough Hemolysis through Day 183**
   - Defined as elevated LDH ≥ 2x ULN (after prior reduction to < 1.5x ULN on therapy) and any symptom or sign of intravascular hemolysis[a]
4. **Proportion of Patients with Stabilized Hemoglobin (Hb) through Day 183**
   - Defined as avoidance of a decrease of ≥ 2 g/dL in Hb level from BL in the absence of transfusion

[a]Breakthrough hemolysis defined as at least one new or worsening symptom or sign of intravascular hemolysis (fatigue, hemoglobinuria, abdominal pain, shortness of breath [dyspnea], anemia [hemoglobin < 10 g/dL], major adverse vascular event [MAVE, including thrombosis], dysphagia, or erectile dysfunction) in the presence of elevated LDH ≥ 2 × ULN, after prior LDH reduction to < 1.5 × ULN on therapy.

Non-Inferiority Achieved on Co-Primary Endpoints



	ALXN1210 (N=125)	Eculizumab (N=121)	Treatment Diff (95% CI)
Transfusion Avoidance Diff in Proportion of Patients	73.6	66.1	6.8 (-4.7, 18.1)[a]



	ALXN1210 (N=125)	Eculizumab (N=121)	Odds Ratio (95% CI)[1]
LDH Normalization Odds Ratio	53.6	49.4	1.19 (0.8, 1.77) [b]

▲ = Non-inferiority Margin for the Given Endpoint

Non-inferiority Achieved on Both Co-primary Endpoints: Numerically Favors ALXN1210

Note: CI=Confidence interval; Diff=difference; TA=Transfusion Avoidance; LDH-N=LDH Normalization. OR=Odds ratio Note: For endpoint TA, Diff (95% CI) are based on estimated differences in percent with 95% CI. For LDH-N, adjusted prevalence within each treatment are displayed. Treatment difference is estimated for ALXN1210-eculizumab. [1] To calculate the OR of ALXN1210 relative to eculizumab from the displayed adjusted prevalence rates, divide the odds of LDH-N on ALXN1210 (0.536/(1-0.536)) by the odds of LDH-N on eculizumab (0.494/(1-0.494))

[a]Difference in proportion of patients; [b] Odds ratio

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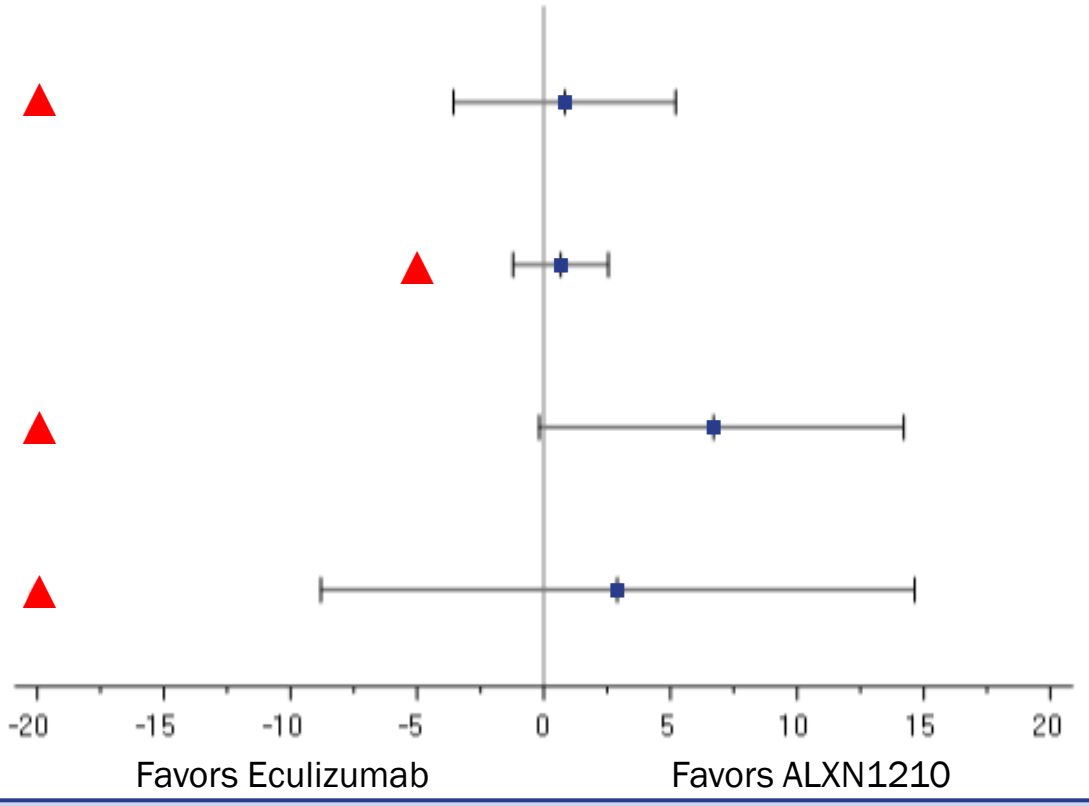
Non-Inferiority Achieved on All 4 Key Secondary Endpoints



	ALXN1210 (N=125)	Eculizumab (N=121)	Diff (95% CI)[1]
LDH Reduction	-76.84	-76.02	0.83 (-3.6, 5.2)[2,a]
FACIT-Fatigue Improvement	7.07	6.40	0.67 (-1.2, 2.6)[a]
Rate of Breakthrough Hemolysis	4.0	10.7	6.7 (-0.18,14.2)[2,b]
Stabilization of Hb levels	68.0	64.5	2.9 (-8.8, 14.6)[b]

▲ = Non-inferiority Margin for the Given Endpoint

Non-inferiority Achieved on All Key Secondary Endpoints: Numerically Favors ALXN1210

Note: CI=Confidence interval; Diff=difference; [1]For endpoints LDH Reduction vs. BL, Reduction in Breakthrough Hemolysis, and Stabilization of Hb levels (95% CI) are based on estimated differences in percent with 95% CI. For FACIT–Fatigue, Diff (95% CI) are based on estimated differences in change from baseline.
[2] Treatment difference is estimated for ALXN1210-eculizimab except for LDH Reduction vs BL and Reduction in Breakthrough Hemolysis where treatment difference is based on eculizumab-ALXN1210.
[a]Difference in change vs. Baseline; [b]Difference in proportion of patients;
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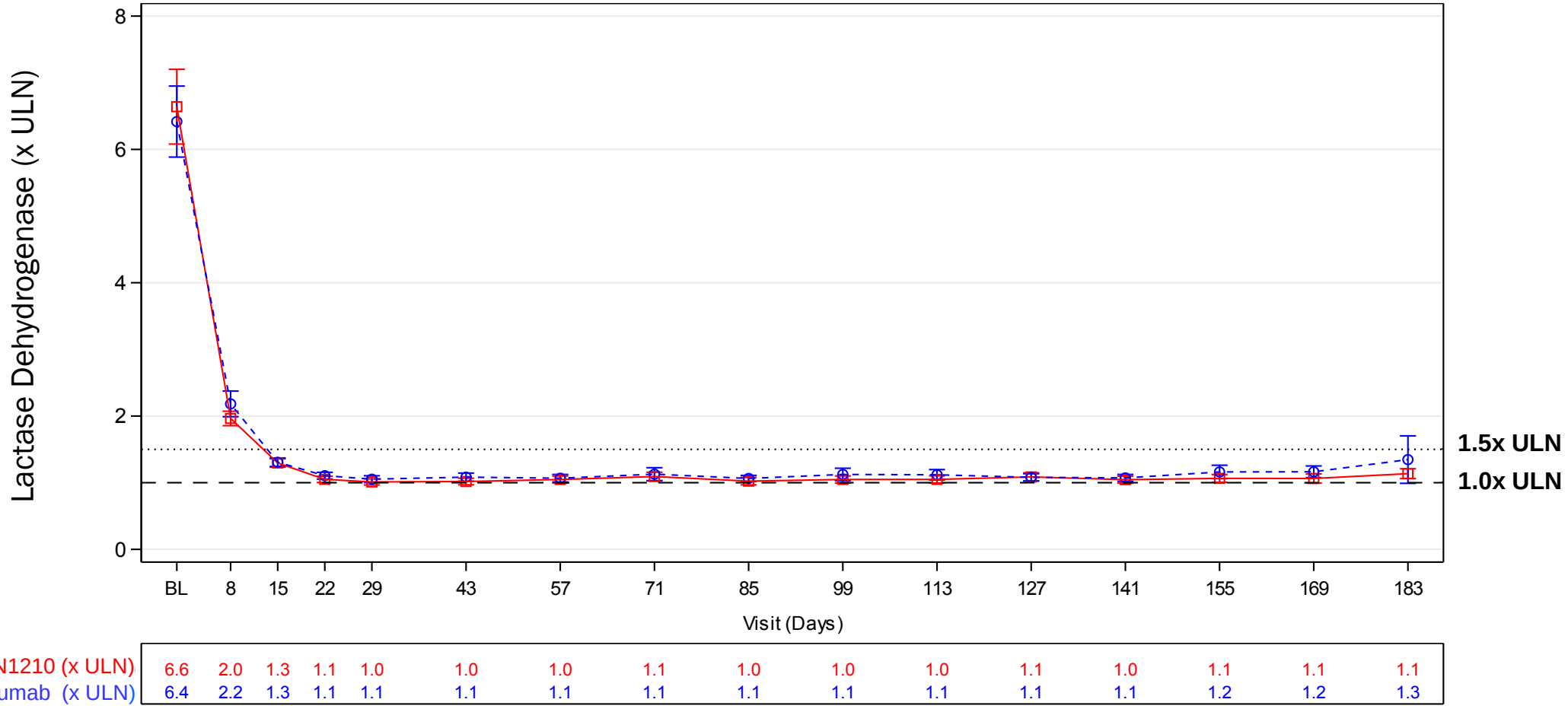
ALXN-1210 Results in Rapid and Sustained Reductions in LDH with Mean Levels ~1x ULN



	BL	8	15	22	29	43	57	71	85	99	113	127	141	155	169	183
Mean ALXN1210 (x ULN)	6.6	2.0	1.3	1.1	1.0	1.0	1.0	1.1	1.0	1.0	1.0	1.1	1.0	1.1	1.1	1.1
Mean Eculizumab (x ULN)	6.4	2.2	1.3	1.1	1.1	1.1	1.1	1.1	1.1	1.1	1.1	1.1	1.1	1.2	1.2	1.3

Note: BL=Baseline. ULN=upper limit of normal. The ULN for LDH is 246 U/L.

Note: Baseline is defined as the average of all available assessments from the central laboratory prior to first study drug dose.

Note: The dashed horizontal line indicates 1 x ULN and the dotted horizontal line represents 1.5 x ULN.

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Safety and Treatment Compliance Summary

Safety	ALXN1210 (N=125)	Soliris® (N=121)	Total (N=246)
	N (%)	N (%)	N (%)
Any Adverse Event (AE)	110 (88.0%)	105 (86.8%)	215 (87.4%)
Most Common AEs (>10%)			
Headache	45 (36.0%)	40 (33.1%)	85 (34.6%)
Nasopharyngitis	11 (8.8%)	18 (14.9%)	29 (11.8%)
Upper respiratory tract infections	13 (10.4%)	7 (5.8%)	20 (8.1%)
Pyrexia	6 (4.8%)	13 (10.7%)	19 (7.7%)
Any Serious Adverse Event (SAE)	11 (8.8%)	9 (7.4%)	20 (8.1%)
Meningococcal Infections	0 (0.0%)	0 (0.0%)	0 (0.0%)
Death	0 (0.0%)	1 (0.8%)[1]	1 (0.4%)
AE Leading to Withdrawal of Study Drug	0 (0.0%)	1 (0.8%)[1]	1 (0.4%)
SAE Leading to Withdrawal of Study Drug	0 (0.0%)	1 (0.8%)[1]	1 (0.4%)

100% treatment compliance for ALXN1210 and 99.95% for Soliris®

ALXN1210 was Well-Tolerated and had a Safety Profile Similar to Soliris®

[1]One patient in the Soliris® arm died from lung cancer (unrelated to Soliris® treatment) during the extension phase of the study.

Closing Remarks

Ludwig Hantson, Ph.D.
Chief Executive Officer

Summary & Next Steps

- Very pleased with the success of ALXN1210 in the pivotal head-to-head study vs. Soliris®
  - Achieved non-inferiority on both co-primary and all 4 key secondary endpoints, with numeric results favoring ALXN1210
  - ALXN1210 safety profile consistent with that of Soliris®
  - Every 8 week dosing could be a meaningful improvement for patients with PNH
- Results from the Phase 3 PNH switch study expected in 2Q18
- Phase 3 study in aHUS enrolling with data expected in 4Q18
- Regulatory filings planned for PNH in the U.S., EU and Japan in 2H18
- Ambition to establish ALXN1210 as the new standard of care in PNH

Q&A

ALXN1210 PNH Naïve Phase 3 Study Results

Appendix

ALXN1210 PNH Naïve Phase 3 Study Results

Efficacy Results Summary

	Endpoint	Treatment effect [95% CI: LB, UB]		Statistic for Tx Comparison	Treatment Difference ALXN1210 vs. Soliris [95% CI: LB, UB]	Non-inferiority	
		ALXN1210 (n=125)	Soliris® (n=121)			Requirement	Achieved*
Co-Primary	**Transfusion Avoidance**	**73.6%** (65.9%, 81.3%)	**66.1%** (57.7%,74.6%)	Difference in Rate	**6.8%** (-4.7%,18.1%)[a]	LB > -20%	Yes
	LDH Normalization	**53.6%** (45.9%, 61.2%)	**49.4%** (41.7%, 57.0%)	Odds Ratio	**1.19** (0.80, 1.77)[b]	LB > 0.39	Yes
Secondary	**Change in LDH Levels**	**-76.8%** (-80.0%, -73.7%)	**-76.0%** (-79.2%, -72.8%)	Difference in % Change from Baseline	**-0.83%** (-5.2%, 3.6%)[c]	UB < 20%	Yes
	Improvement in FACIT Scale	**7.1** (5.6, 8.6)	**6.4** (4.9, 8.0)	Difference in Change from Baseline	**0.67** (-1.2, 2.6)[c]	LB > -5.0	Yes
	Breakthrough Hemolysis	**4.0%** (0.6%,7.4%)	**10.7%** (5.2%, 16.3%)	Difference in Rate	**-6.7%** (-14.2%, 0.18%)[a]	UB < 20%	Yes
	Stabilization of Hb levels	**68.0%** (59.8%,76.2%)	**64.5%** (55.9%, 73.0%)	Difference in Rate	**2.9%** (-8.8%, 14.6%)[a]	LB > -20%	Yes

LDH: lactate dehydrogenase; FACIT: Functional Assessment of Chronic Illness Therapy; Hb: hemoglobin; CI: confidence interval; LB: lower bound; UB: upper bound

*Non-inferiority is achieved if the LB or UB of the 95% CI of the treatment difference meets the pre-defined requirement. Since non-inferiority was achieved across both co-primary and all four key secondary endpoints, the protocol allowed for superiority testing. Testing for superiority followed a closed-testing procedure, using a 2-sided 0.05 test for each parameter. Testing followed the pre-specified order per protocol: Breakthrough hemolysis, LDH reduction vs. Baseline, LDH normalization, Reduction in FACIT scale, Stabilization of Hb levels, TA achievement. As breakthrough hemolysis did not achieve statistical significance (p-value = 0.074), no other endpoints were tested.

[a]Difference in proportion of patients; [b] Odds ratio; [c] Difference in change vs. Baseline

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