



Topline results from the Phase IIb monotherapy study of vobarilizumab, ALX-0061 (anti-IL-6R), in patients with moderate to severe RA

7 July 2016

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Vobarilizumab Phase IIb RA monotherapy study



Participants on the call



Dr Edwin Moses

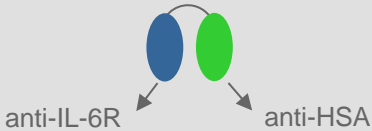
CEO



Dr Robert K. Zeldin

CMO

Unique half-life extended Nanobody product

Features	Potential benefits
Small (26kD) 	<ul style="list-style-type: none">• Penetrates faster and more effectively into tissues
Targets human serum albumin	<ul style="list-style-type: none">• Prolongs half-life• Improved trafficking to inflamed tissue
Monovalent binding	<ul style="list-style-type: none">• Avoids target cross-linking
Preferential binding of soluble vs. membrane bound IL-6R	<ul style="list-style-type: none">• Superior benefit/risk profile
Strong affinity to soluble IL-6R	<ul style="list-style-type: none">• Fast target engagement resulting in fast onset of action
Tailored PK	<ul style="list-style-type: none">• Extended therapeutic window• Convenient dosing and scheduling

Anti-IL-6R Nanobody – ALX-0061, vobarilizumab



Potential best-in-class treatment for Rheumatoid Arthritis (RA)

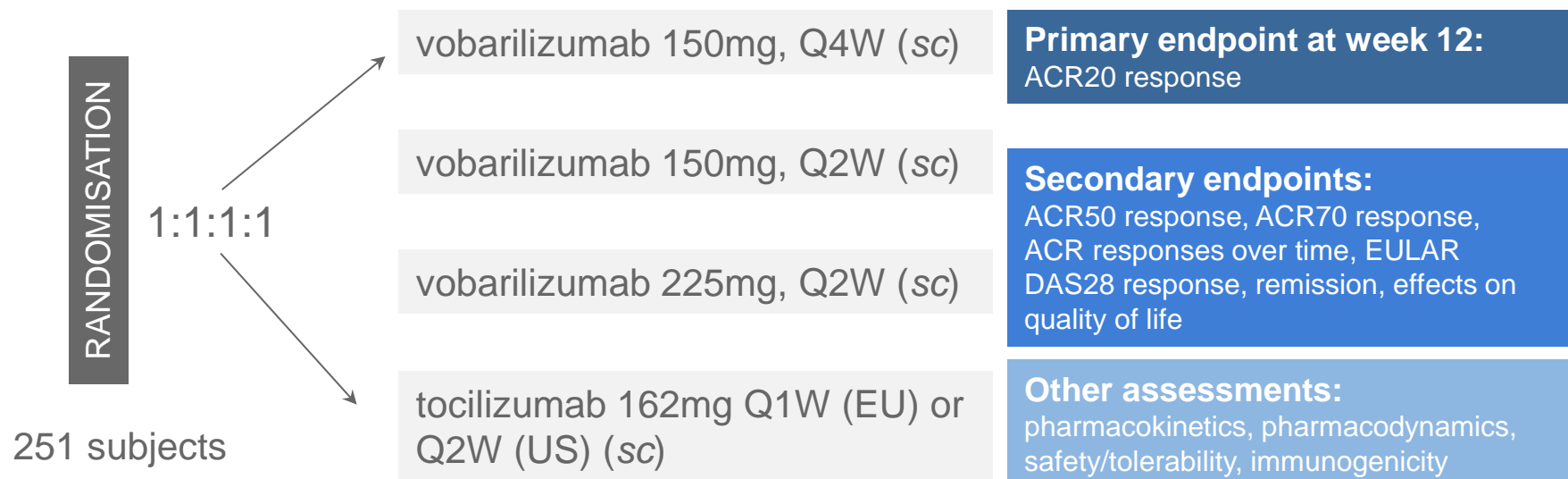
- **Best-in-class potential** for the treatment of RA
- Global option licensing deal with **AbbVie**
- Completed recruitment of 251 patients in a **RA monotherapy** study and 345 patients in a **RA combination therapy** study
- **Open-label extension** study ongoing in **RA** patients
- **Phase II** study in **SLE** patients ongoing



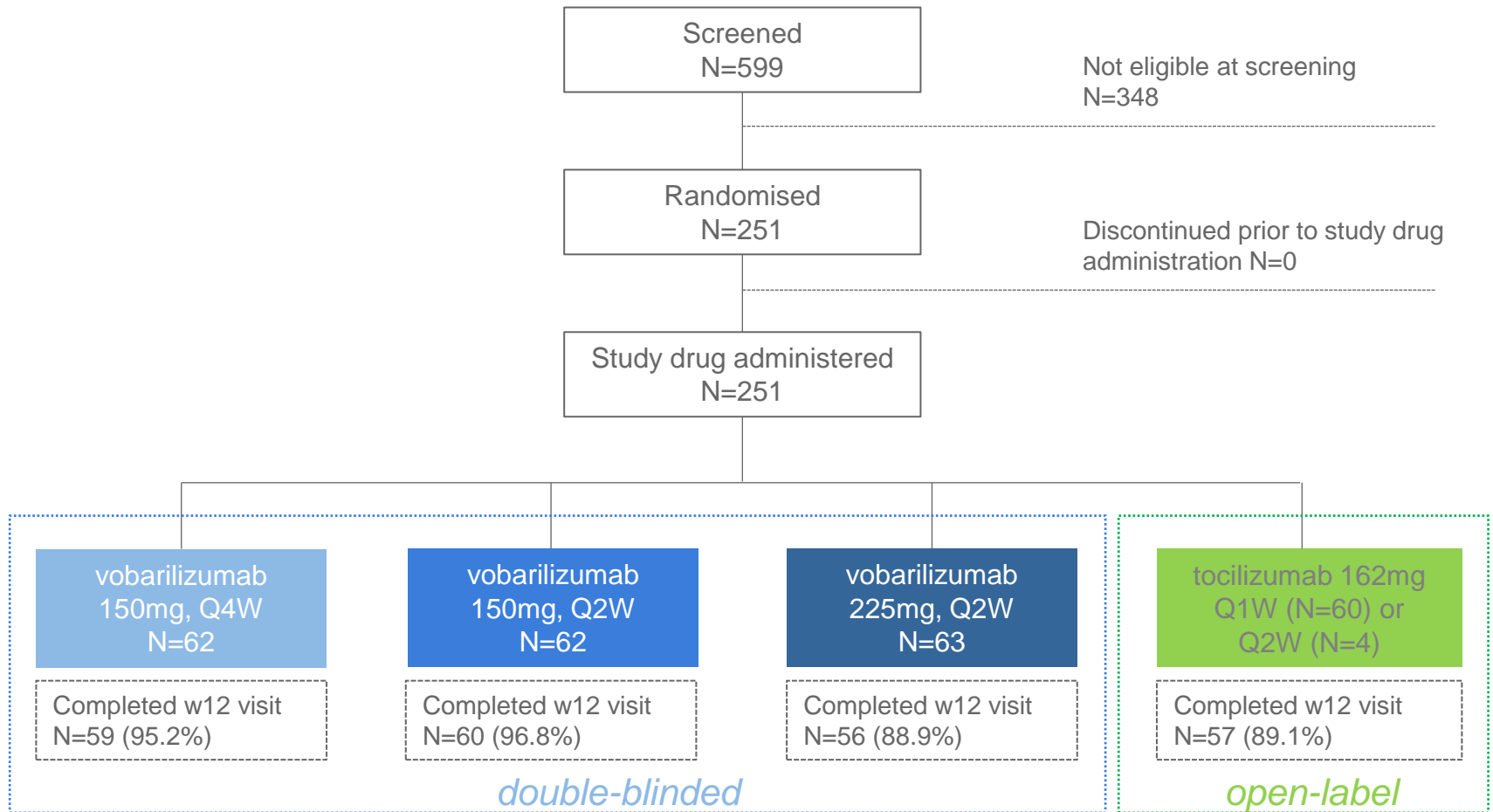
Vobarilizumab

Phase IIb RA monotherapy study in 251 RA patients

- Adult subjects with moderate to severe RA who are intolerant to MTX or for whom continued MTX is inappropriate
- Open-label tocilizumab* arm to obtain parallel descriptive information on efficacy and safety
- Randomised, double-blind 12 week study in the US, Europe, and Latin America
- Recruitment from April 2015 to February 2016



Patient disposition



A very high proportion of vobarilizumab treated patients completed the study

Baseline demographics and disease activity – ITT population

Mean (SD)	vobarilizumab 150mg, Q4W N=62	vobarilizumab 150mg, Q2W N=62	vobarilizumab 225mg, Q2W N=63	tocilizumab 162mg Q1W, N=60 or Q2W, N=4
Age, years	53.0 (12.3)	51.2 (12.1)	51.3 (11.8)	50.0 (12.3)
Females (%)	79.0	85.5	85.7	87.5
Duration of RA, years	8.0 (7.4)	8.4 (6.7)	7.7 (8.0)	6.8 (5.7)
TJC68	27.9 (16.0)	28.1 (14.3)	25.8 (13.5)	27.3 (13.1)
SJC66	14.4 (7.7)	17.1 (9.5)	17.3 (8.5)	17.3 (9.8)
CRP, mg/L	17.7 (19.9)	23.6 (22.9)	33.5 (41.6)	22.0 (20.7)
DAS28 _{CRP}	5.9 (0.9)	6.2 (0.9)	6.1 (1.0)	6.2 (0.9)
HAQ-DI score	1.6 (0.7)	1.8 (0.7)	1.8 (0.7)	1.7 (0.8)

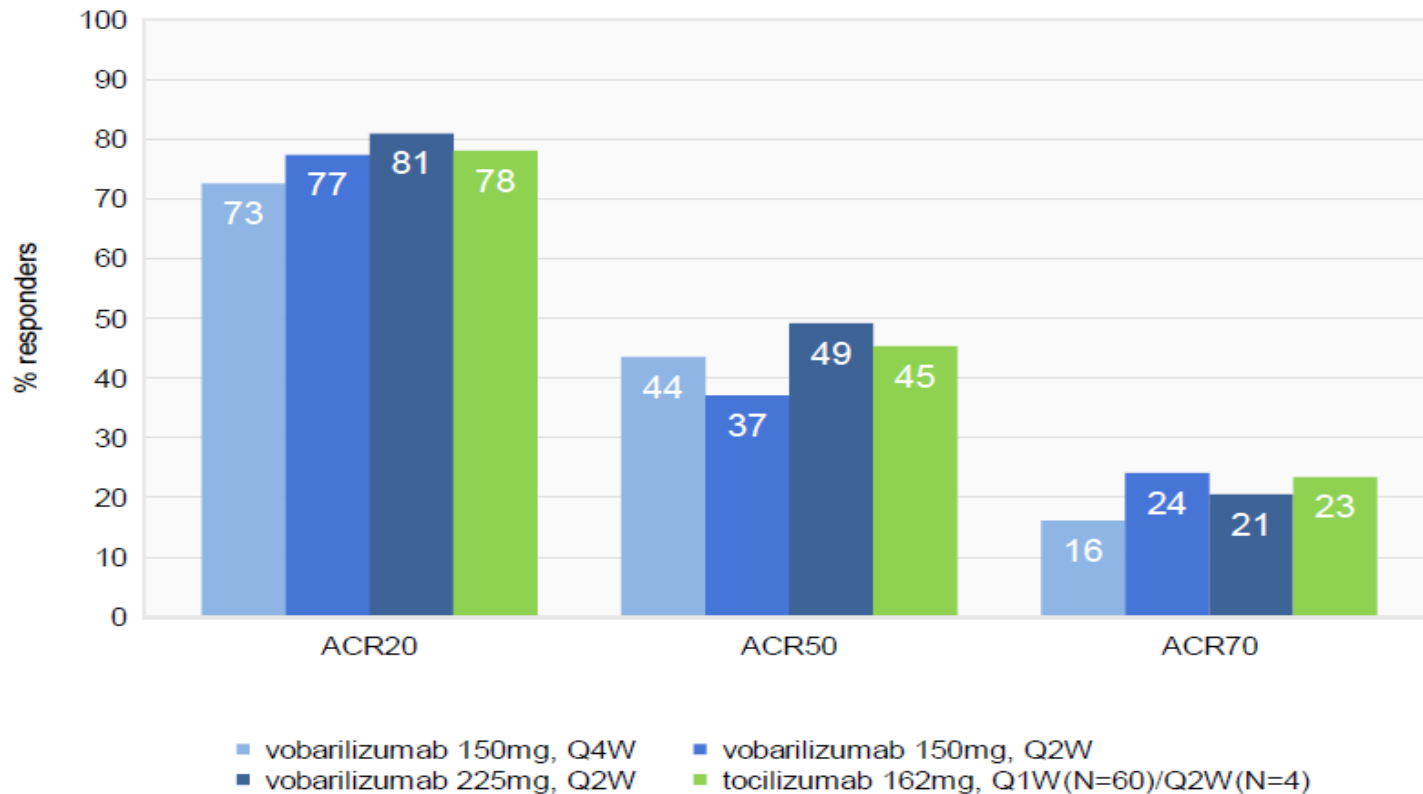
Baseline demographics reflective of a typical RA population with similar disease activity across the groups

Vobarilizumab Phase IIb RA monotherapy study



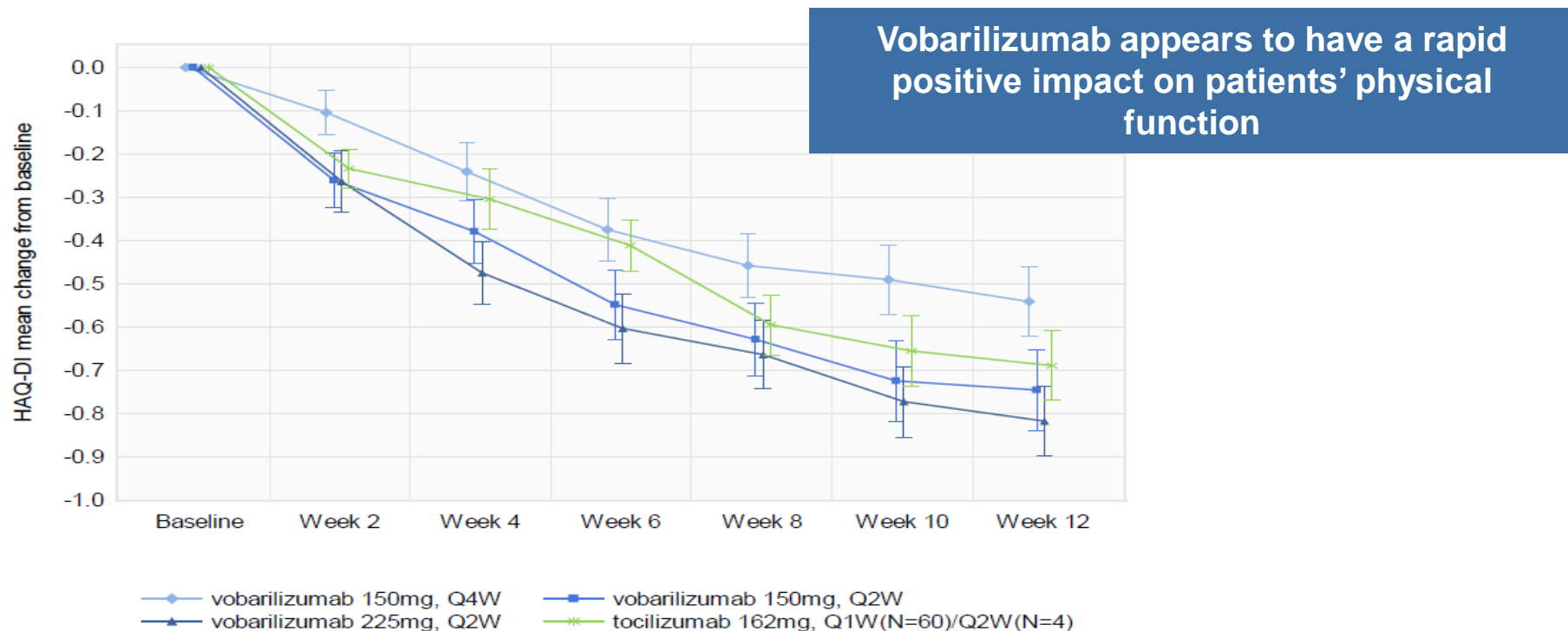
ACR20/50/70 responses at week 12 – ITT population

- Primary endpoint: ACR20 response at week 12



Vobarilizumab appears to be very effective with less frequent administration than tocilizumab

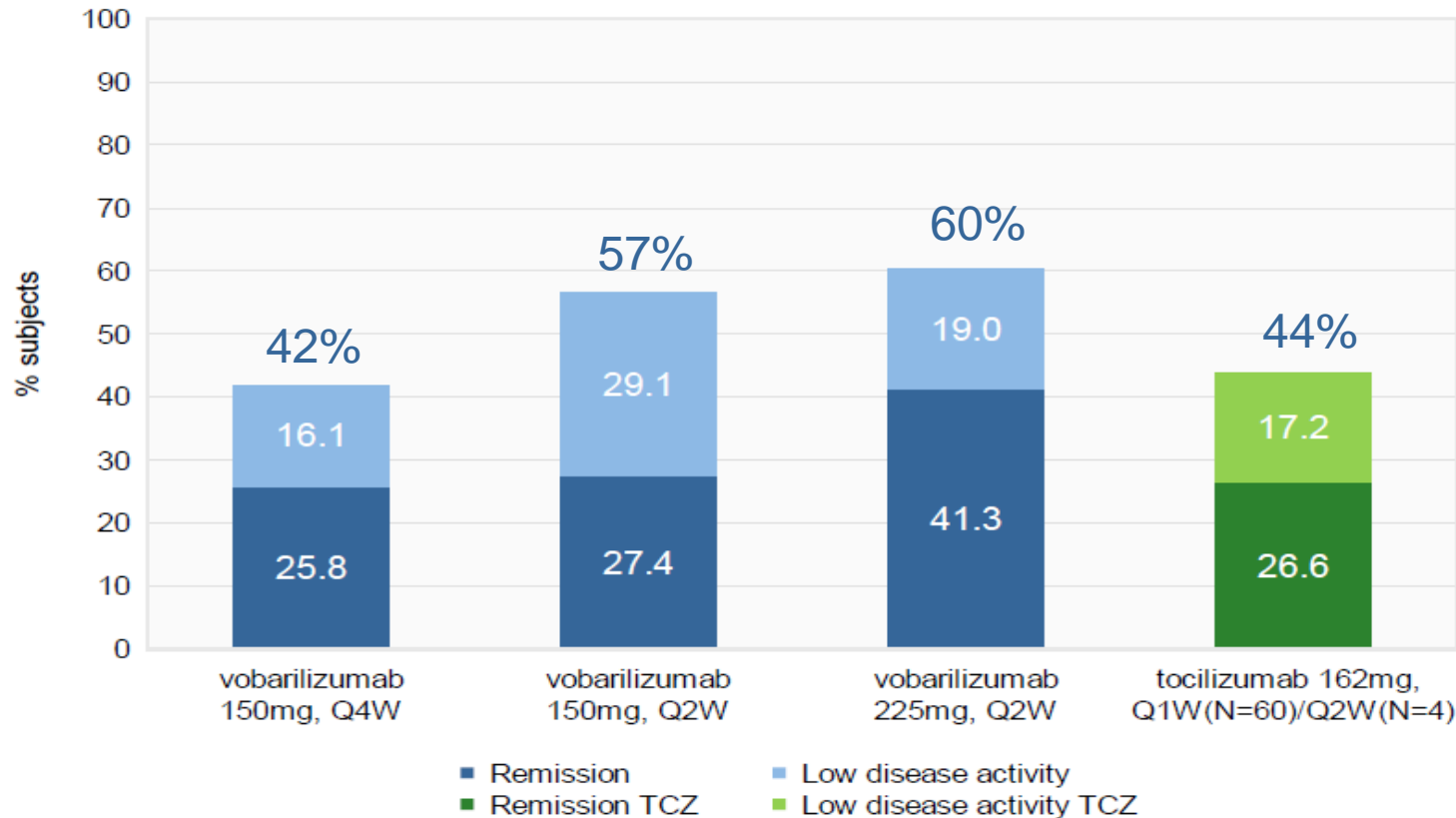
HAQ-DI score at week 12 and change from baseline – ITT population



	vobarilizumab 150mg, Q4W N=62	vobarilizumab 150mg, Q2W N=62	vobarilizumab 225mg, Q2W N=63	tocilizumab 162mg Q1W, N=60 or Q2W, N=4
% with a clinically meaningful improvement in HAQ-DI score [*] (≥0.25) at week 12	65%	68%	71%	72%
Absolute change from baseline at week 12 (mean)	-0.54	-0.75	-0.82	-0.69

^{*} Wolfe F. *et al*, Arthritis & Rheumatism, Vol. 42, No. 9, September 1999, pp 1797–1808

Remission and low disease activity at week 12* – ITT population



Vobarilizumab induces either clinical remission or low disease activity in up to 60% of patients at week 12

* Remission: $DAS28_{CRP} < 2.6$; low disease activity: $2.6 \leq DAS28_{CRP} \leq 3.2$

Interim safety results at week 12

Number of subjects (%) with treatment-emergent adverse events (TEAE)	vobarilizumab 150mg, Q4W N=62	vobarilizumab 150mg, Q2W N=62	vobarilizumab 225mg, Q2W N=63	tocilizumab 162mg Q1W (N=60) or Q2W (N=4)
Any TEAE	34 (54.8)	33 (53.2)	31 (49.2)	31 (48.4)
- treatment-related	21 (33.9)	19 (30.6)	21 (33.3)	20 (31.3)
- leading to study drug discontinuation	1 (1.6)	1 (1.6)	2 (3.2)	4 (6.3)
	vobarilizumab, all doses N=187			tocilizumab 162mg Q1W (N=60) or Q2W (N=4)
Any serious TEAE	1 (0.5)			2 (3.1)
- treatment-related	1 (0.5)			2 (3.1)
- leading to death	0			0

Favourable safety profile for vobarilizumab at all doses tested

Safety laboratory abnormalities through week 12

% of subjects	vobarilizumab, all doses N=187	tocilizumab, 162mg Q1W (N=60) or Q2 (N=4)
Aspartate aminotransferase		
>3 – ≤5 x ULN (grade 2)	1.1%	1.6%
>5 – ≤20 x ULN (grade 3)	0.5%	0%
Alanine aminotransferase		
>3 – ≤5 x ULN (grade 2)	0%	0%
>5 – ≤20 x ULN (grade 3)	0.5%	0%
Absolute neutrophil count		
<1,5 to 1,0 x 10 ⁹ /L (grade 2)	8.7%	9.4%
<1,0 to 0,5 x 10 ⁹ /L (grade 3)	1.1%	4.7%
Absolute platelet count		
<75.0 to 50.0 x 10 ⁹ /L (grade 2)	0.5%	1.6%
<50.0 to 25.0 x 10 ⁹ /L (grade 3)	0%	0%

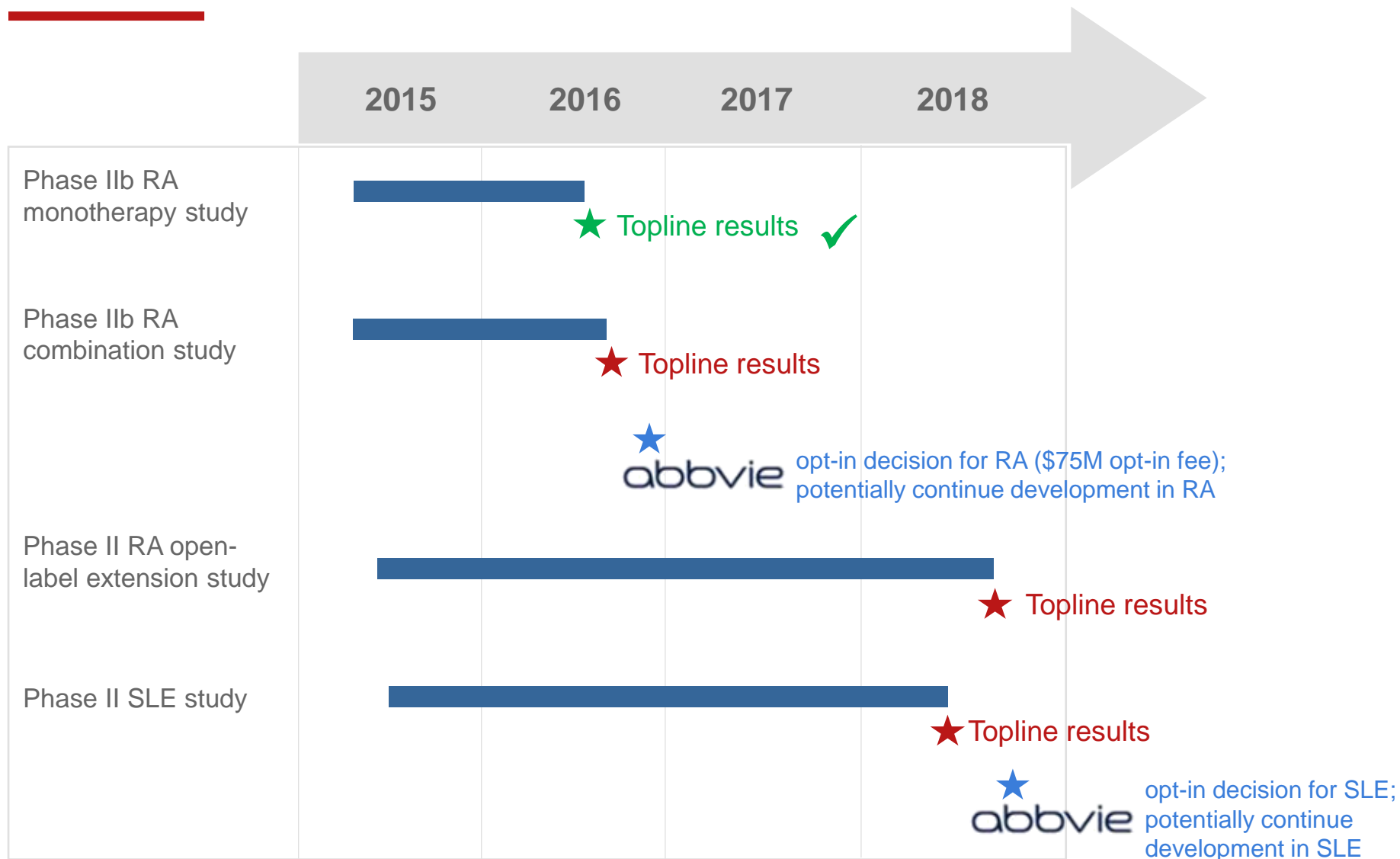
Favourable safety profile confirmed with only infrequent abnormalities observed in laboratory assessments

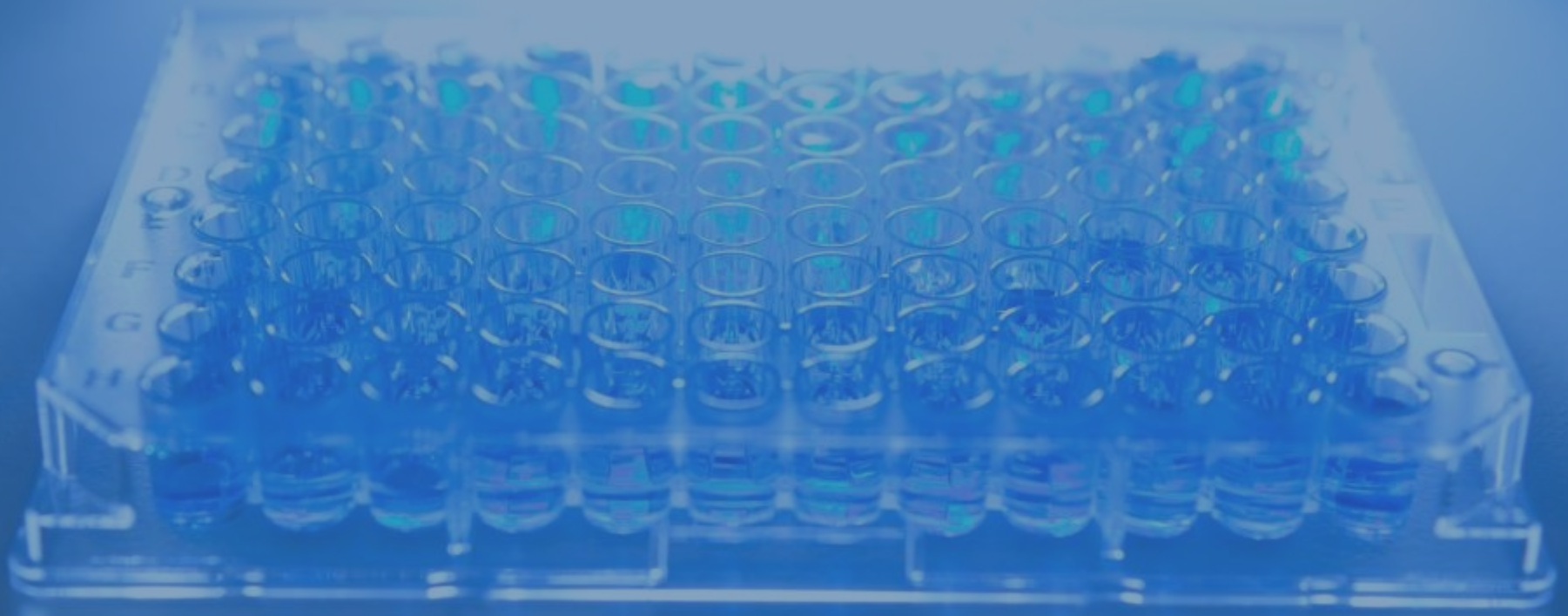
Conclusions from topline results – week 12

- Very encouraging efficacy data for vobarilizumab with ACR20, ACR50 and ACR70 scores of up to 81%, 49% and 24%, respectively
- Rapid improvement in patients' physical function based on the change from baseline in HAQ-DI score
- Robust DAS28_{CRP} data for vobarilizumab with up to 60% of patients in either clinical remission or low disease activity at week 12 compared to 44% for open-label tocilizumab
- Favourable safety profile at all administered doses

Promising efficacy and safety profile
Final data analysis ongoing

Key upcoming catalysts





Questions

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