



# Vivacity-MG Phase 2 Interim Analysis Topline Results

Investor and Analyst Conference Call  
June 15, 2020

## **Introduction**

- Craig Wheeler, President and Chief Executive Officer

## **Trial Design and Overview of Results**

- Santiago Arroyo, SVP and Chief Medical Officer

## **Closing Remarks**

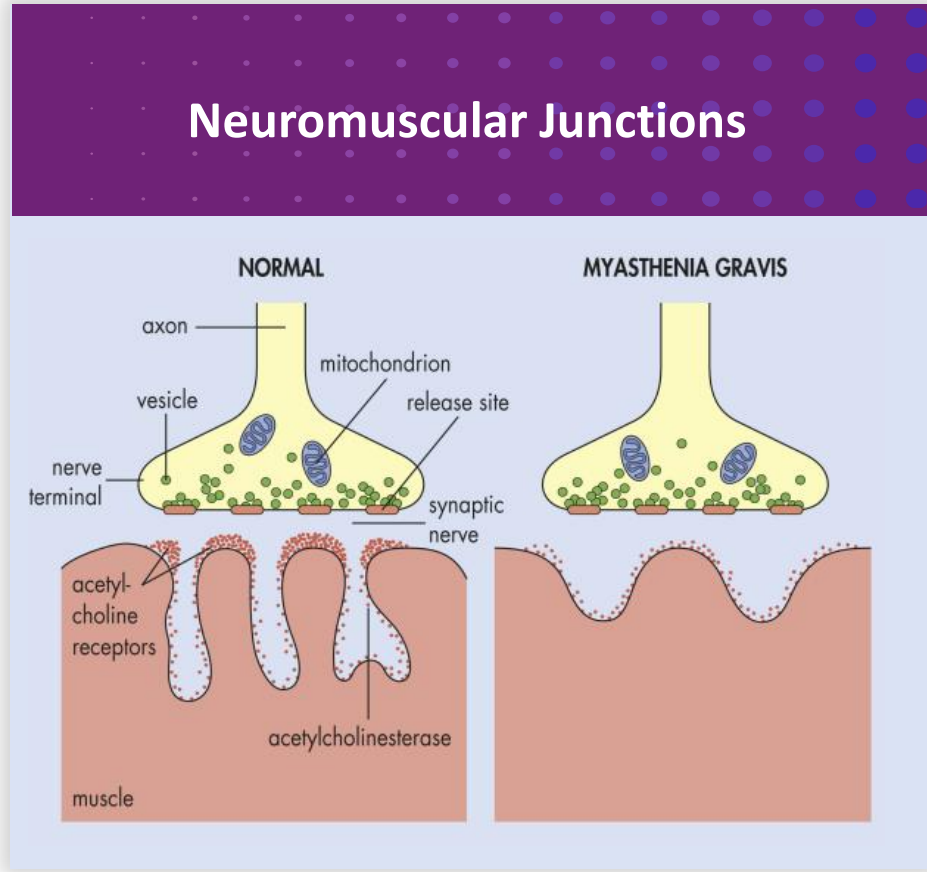
- Craig Wheeler, President and Chief Executive Officer

## **Question & Answer Session**

# Forward Looking Statements

Statements in this presentation regarding management's future expectations, beliefs, intentions, goals, strategies, plans or prospects, are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including but not limited to statements about our novel drug candidates for immune-mediated disorders, which include M281; the design, timing, enrollment, strategy and goals of clinical trials and the availability, timing and announcement of data and results; the use, efficacy, safety, potency, dosing, tolerability, convenience, differentiation and commercial potential of our products and product candidates, including their potential as best-in-class agents; and our development timelines. Forward-looking statements may be identified by words such as “anticipate” “believe,” “continue,” “expect”, “intend” “plan to,” “objectives”, “building”, “developing”, “potential,” “will,” and other similar words or expressions, or the negative of these words or similar words or expressions. Such forward-looking statements involve known and unknown risks, uncertainties and other important factors, including final and quality controlled verification of interim data and the related analyses; the impact of the COVID-19 pandemic on the status, enrollment, timing and results of our clinical trials, the supply of our manufactured drug materials and our business; the unpredictable nature of early stage development efforts for our product candidates; safety, efficacy or tolerability problems with our product candidates; unexpected adverse clinical trial results; and those referred to under the section "Risk Factors" in the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2020 filed with the Securities and Exchange Commission, as well as other documents that may be filed by the Company from time to time with the Securities and Exchange Commission. As a result of such risks, uncertainties and factors, the Company's actual results may differ materially from any future results, performance or achievements discussed in or implied by the forward-looking statements contained herein. The Company is providing the information in this presentation as of this date and assumes no obligations to update the information included in this presentation or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

# Myasthenia Gravis, a Rare Autoimmune Neuromuscular Disease



- Caused by circulating autoantibodies, most commonly against acetylcholine receptors (AChR) or the muscle-specific receptor tyrosine kinase (MuSK)
- Autoantibodies disrupt these receptors at post-synaptic neuromuscular junctions, thus functionally blocking the excitatory action of acetylcholine
- Bimodal age distribution: younger women and older men prototypical patients

# Nipocalimab (M281): Attributes of a potential best-in-class FcRn Antagonist

## Efficacy

Highest IgG reduction observed, >80%

Ability to maintain 100% receptor occupancy drives IgG lowering and ability to maintain low IgG levels



## Safety

Effectorless antibody design minimizes effector function related AEs

Strong safety profile



## Dosing

Dose-dependent IgG reduction

Rapidly infused IV

Weekly SC option



# Nipocalimab was well tolerated, safe and efficacious in patients with gMG



## Efficacy

Nipocalimab demonstrated efficacy at all doses tested

Statistically significant relationship between IgG reduction and clinical benefit



## Safety

Nipocalimab was well tolerated

No infusion related reactions

No clinically relevant changes in albumin or creatine phosphokinase

No AEs leading to discontinuation, severe AEs, or nipocalimab related SAEs



## Dosing

Efficacy with monthly dosing and seen as early as two weeks

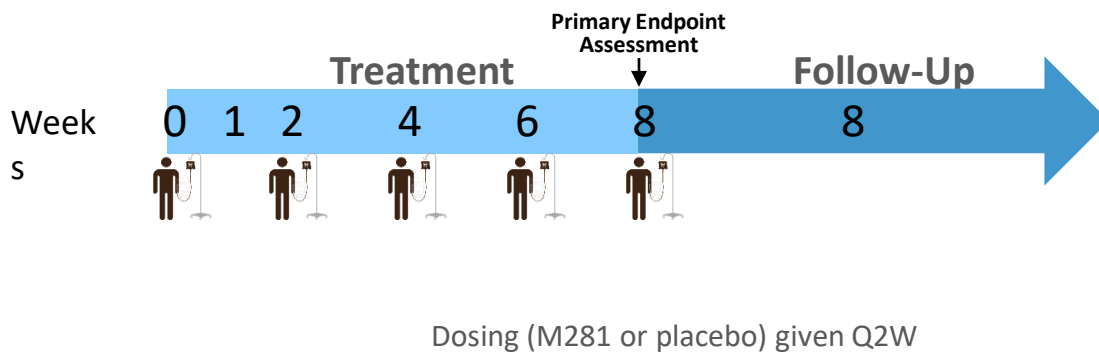
Supports continued clinical development in gMG and subcutaneous formulation dose selection

# Topline Results Represent an Interim Analysis on the Study

- Data set includes all patients through Day 57 (week 8)
- Analysis is for the primary efficacy outcome (MG-ADL change) and safety and laboratory data available up to day 113 (week 16)
- The study was designed to detect a dose responsive efficacy in MG-ADL with at least an 80% power and experiment-wise one-sided type I error of 5%
- COVID-19 did not impact the primary safety and efficacy data
- Expect additional study results to be available later in the year



# Vivacity-MG Phase 2 Study Design



## 68 Subjects Enrolled

### Key Eligibility Criteria:

Age >18 years of age

Documented history of gMG (Class II, III, or IVa); both AChR and anti-MuSK enrolled

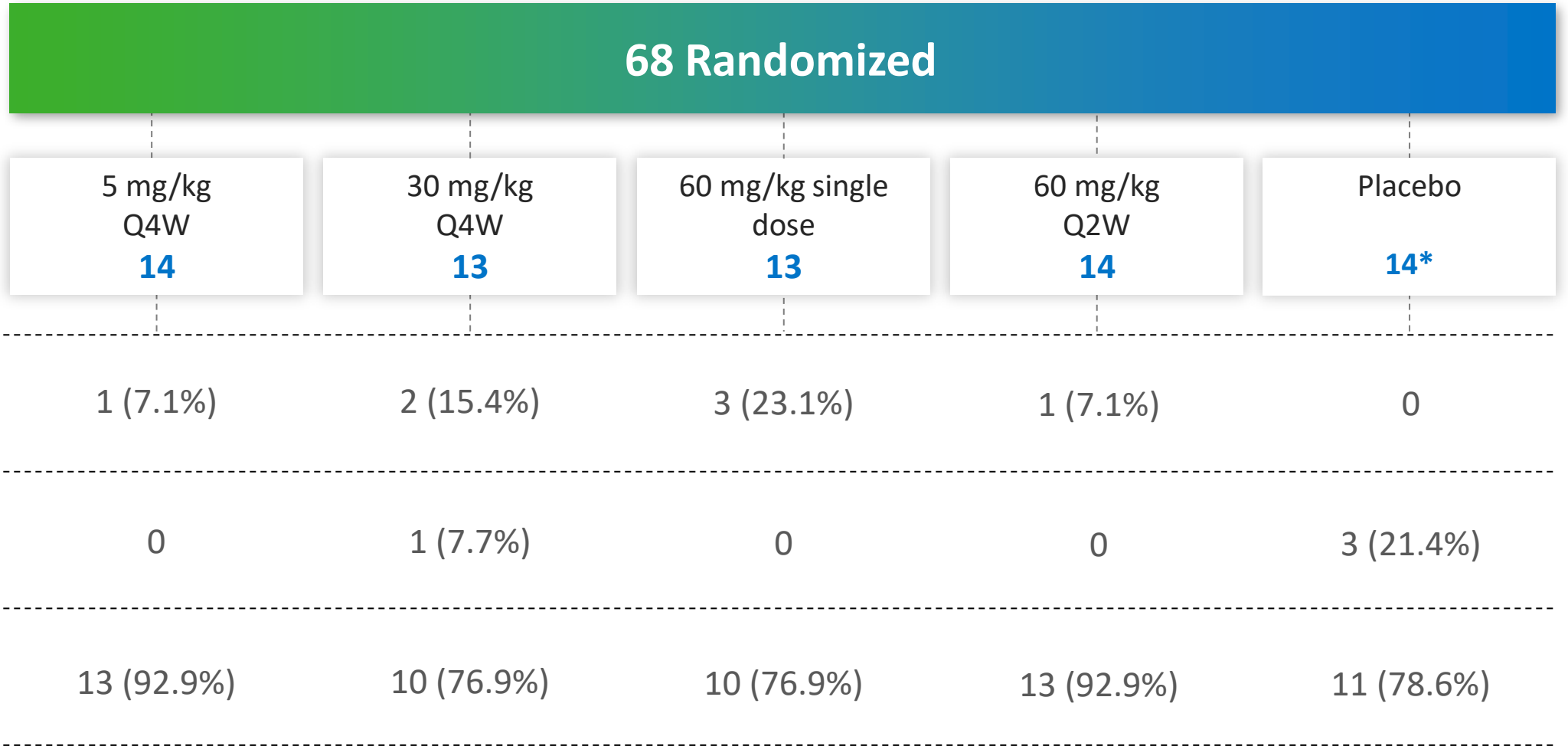
QMG score of  $\geq 12$  & MG-ADL score of  $\geq 4$

On stable MG therapy with no changes during treatment period

No plasmapheresis or IVIG within 6 weeks of randomization



# Vivacity-MG: Subject Disposition



\* 1 patient received 1 dose of study drug, experienced myasthenia worsening 3 days later, did not complete any visit apart from a follow up visit 3 months later and is not included in the mITT analysis

# Baseline Characteristics

	Nipocalimab (n=54)	Placebo (n=13)
Age mean years (SD)	54.3 (17.0)	58.7 (18.2)
Female n (%)	29 (53.7)	7 (53.8)
Time since diagnosis mean months (SD)	80.0 (83.3)	139.9 (114.7)
MG-ADL score mean (SD)	8.0 (2.8)	7.0 (2.7)
QMG score mean (SD)	16.5 (3.5)	17.7 (4.4)
MGFA class n (%)		
II	20 (37.1)	5 (38.5)
III	32 (59.3)	7 (53.9)
IVa	2 (3.7)	1 (7.7)
Anti-AChR positive n (%)	51 (94.4)	12 (92.3)
Anti-MuSK positive n (%)	3 (5.6)	1 (7.7)

# Treatment Emergent Adverse Event Overview

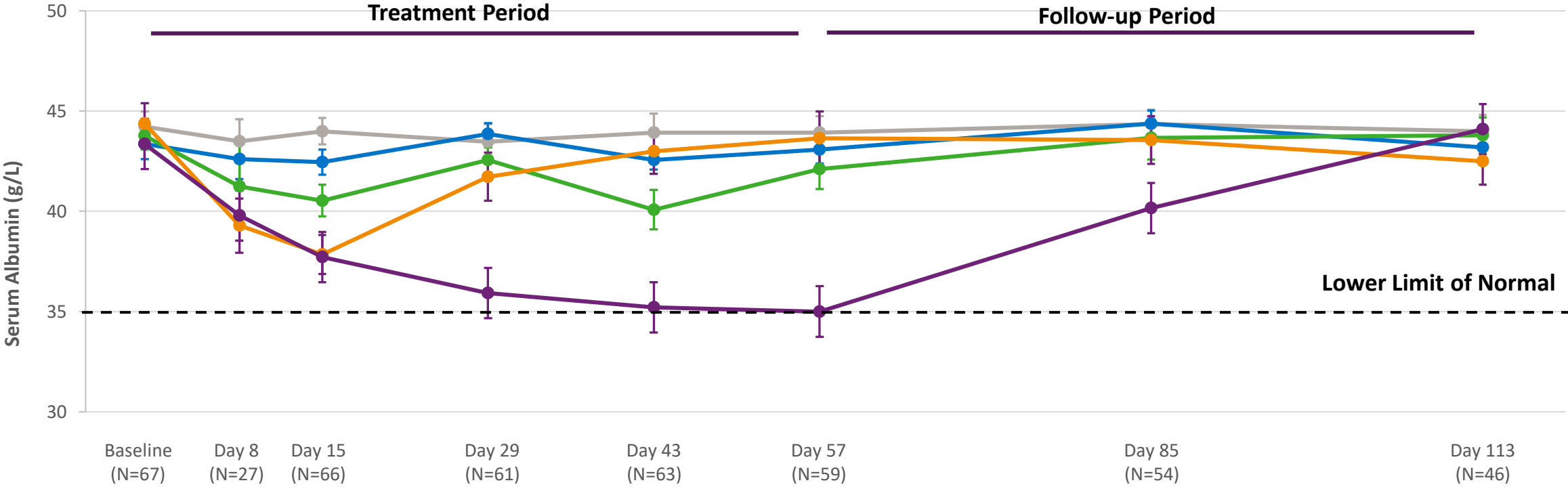
	Nipocalimab (n=54)	Placebo (n=14)
Patients with Adverse Event (AE) <i>n (%)</i>	44 (81.5)	11 (78.6)
Patients with AE grade $\geq 3$ <i>n (%)</i>	0	4 (28.6)
Most frequent AEs <i>n (%)</i>		
Exacerbation of MG	0	2 (14.3)
Headache	6 (11.1)	1 (7.1)
Nasopharyngitis	6 (11.1)	0
Diarrhea	6 (11.1)	1 (7.1)
Patients who discontinued due to AEs <i>n (%)</i>	0	2 (14.3)
Patients with Serious Adverse Event (SAE) <i>n (%)</i>	1 (1.9)*	2 (14.3)*
Patients with AEs deemed related by investigator <i>n (%)</i>	21 (38.9)	1 (7.1)

*There were no clinically relevant CK elevations*

\*SAE deemed unrelated to study drug

# Average Albumin Concentrations Were Within Normal Limits

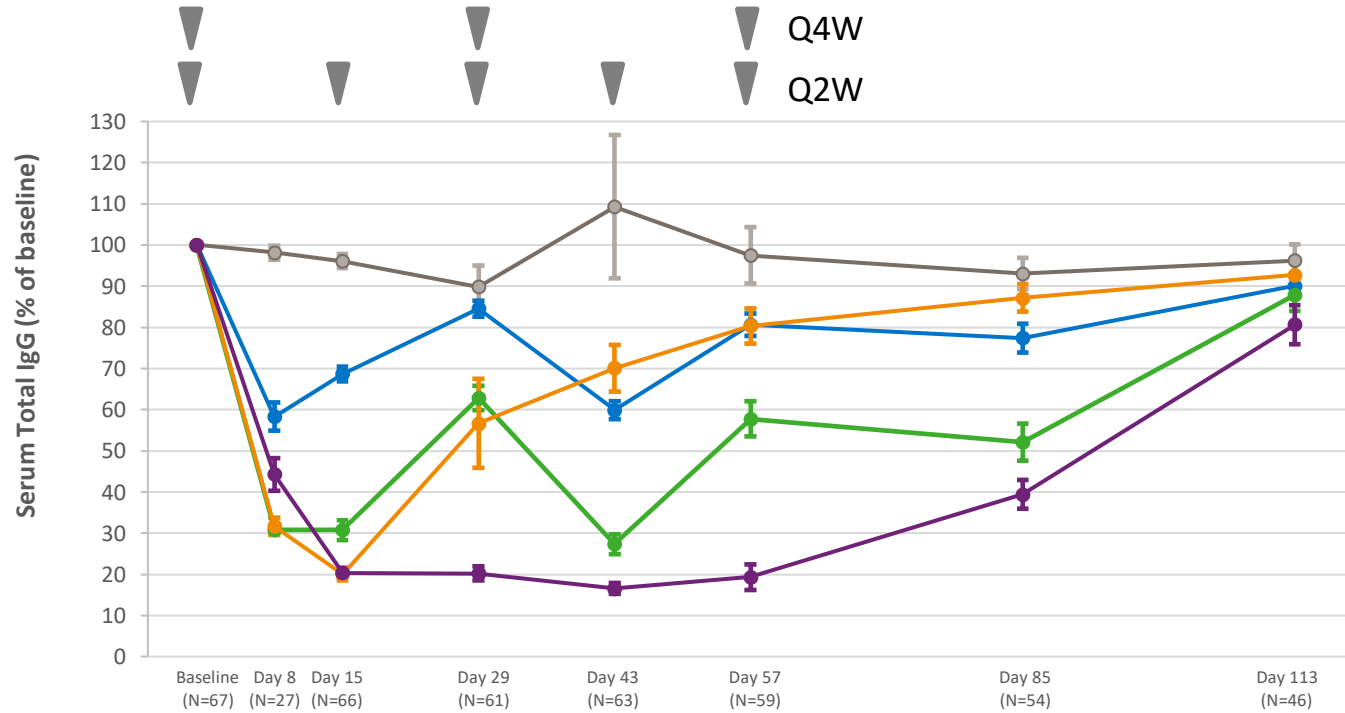
## Serum Albumin Concentrations



Placebo 5 mg/kg Q4W 30 mg/kg Q4W 60 mg/kg single dose 60 mg/kg Q2W

# Rapid and Dose Related Reduction of IgG as Predicted

## Serum Total IgG Concentrations

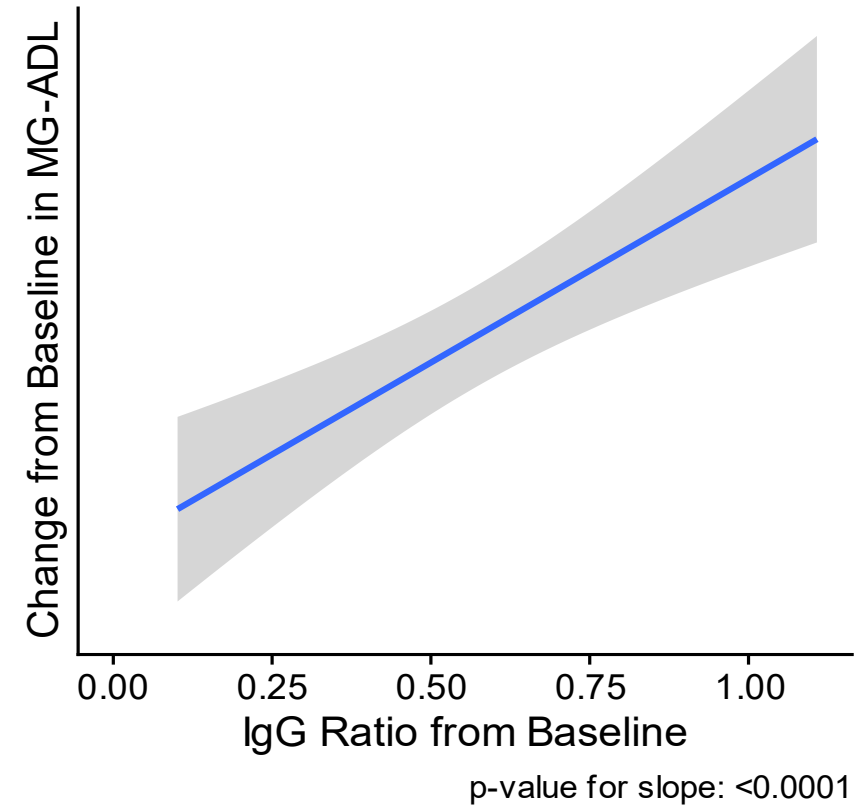


- Dose dependent IgG decreases
- Rapid onset significant lowering of IgG within 1<sup>st</sup> week
- Maximal decrease achieved at 60 mg/kg Q2W
- Similar reductions were seen in:
  - IgG subclasses
  - Anti-AChR antibodies
- No changes in IgA or IgM concentrations

# Robust and Statistically Significant Relationship Between IgG Reduction and Clinical Benefit

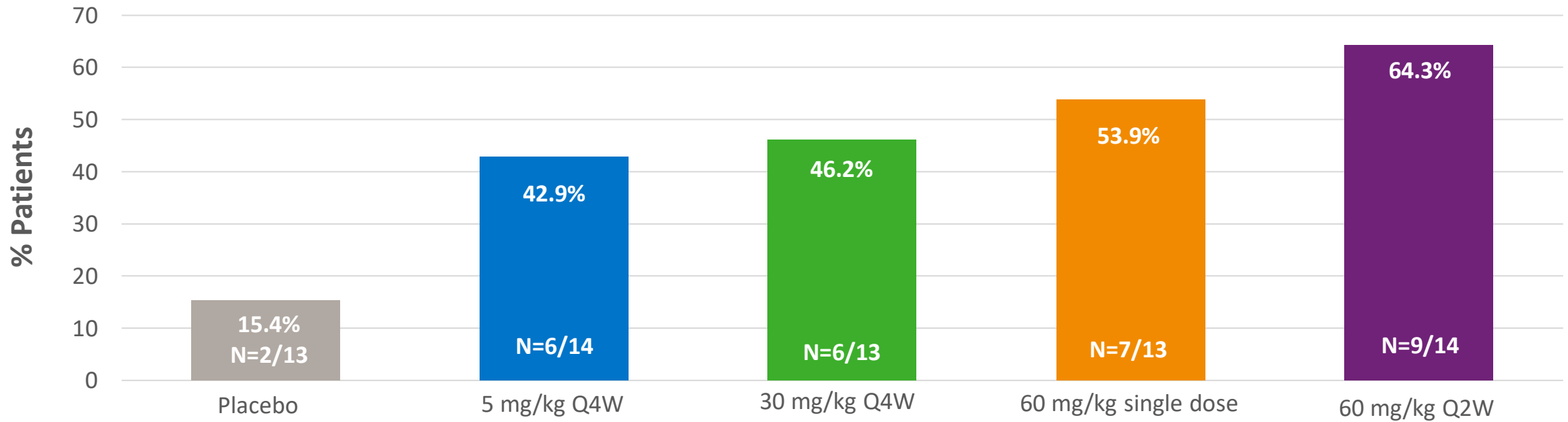
- MG-ADL improvement is highly correlated with serum IgG reduction ( $p < 0.0001$ )
- MG-ADL and Anti-AChR receptor binding antibodies are also highly correlated ( $p < 0.0001$ )

## Comparison of MG-ADL Score and IgG Levels



# Durable MG-ADL Responses at All Doses

Pooled nipocalimab arms showed a 51.9% durable MG-ADL response vs 15.4% in placebo (p-value: 0.017)

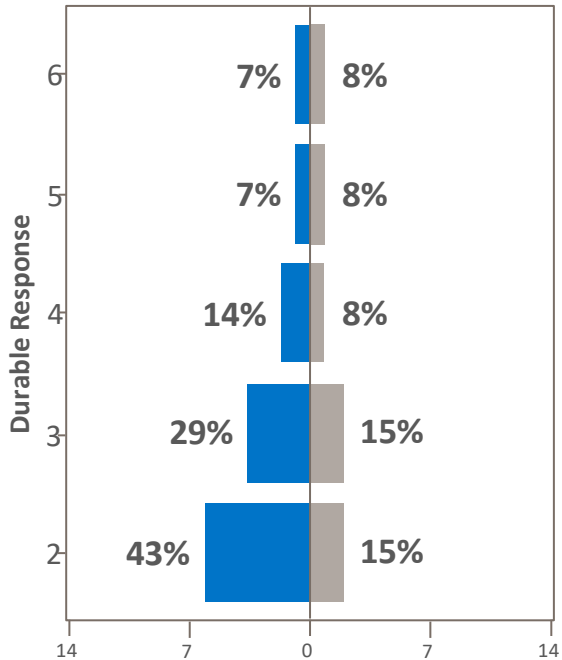


<b>Difference vs Placebo:</b>	27.5%	30.8%	38.5%	48.9%
<b>p-value:</b>	0.1044	0.1008	0.0484	0.0092

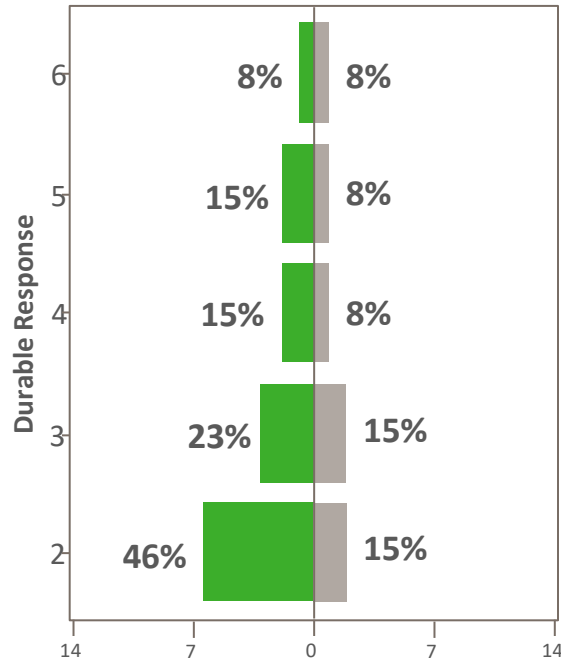
Durable response is defined as improvement in MG-ADL  $\geq 2$  points for at least 4 consecutive weeks during the 1st 8 weeks; p-values are one sided

# Significant Durable Reductions in MG-ADL Across All Doses

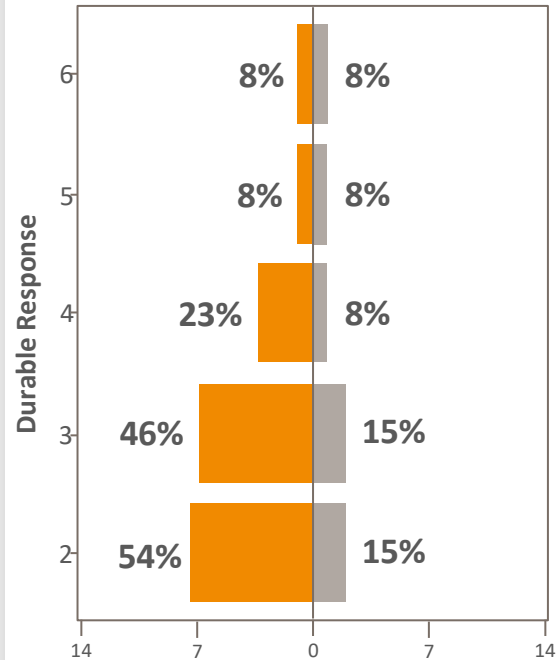
## 5 mg/kg Q4W



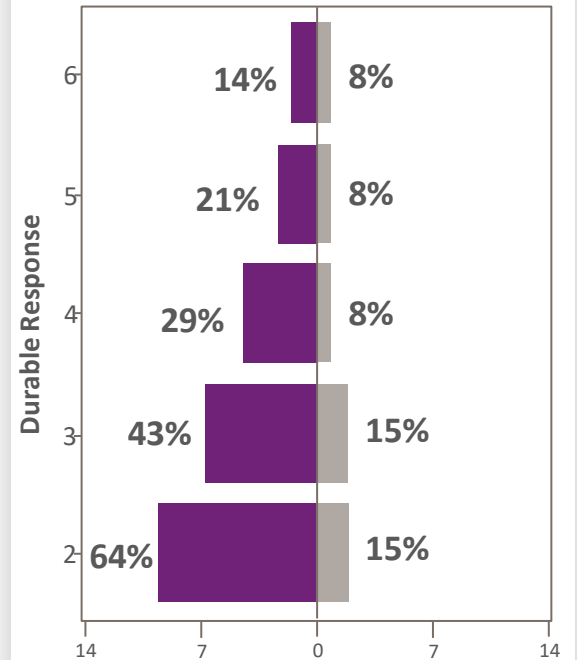
## 30 mg/kg Q4W



## 60 mg/kg single dose



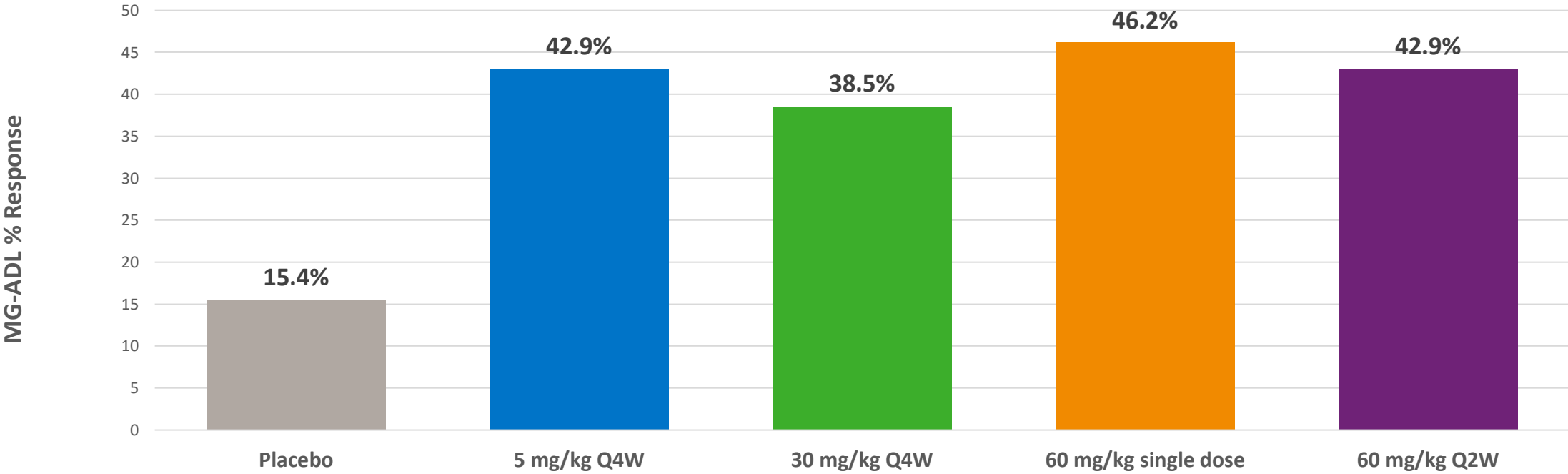
## 60 mg/kg Q2W



Placebo
  M281 5 mg/kg Q4W
  M281 30 mg/kg Q4W
  M281 60 mg/kg single dose
  M281 60 mg/kg Q2W

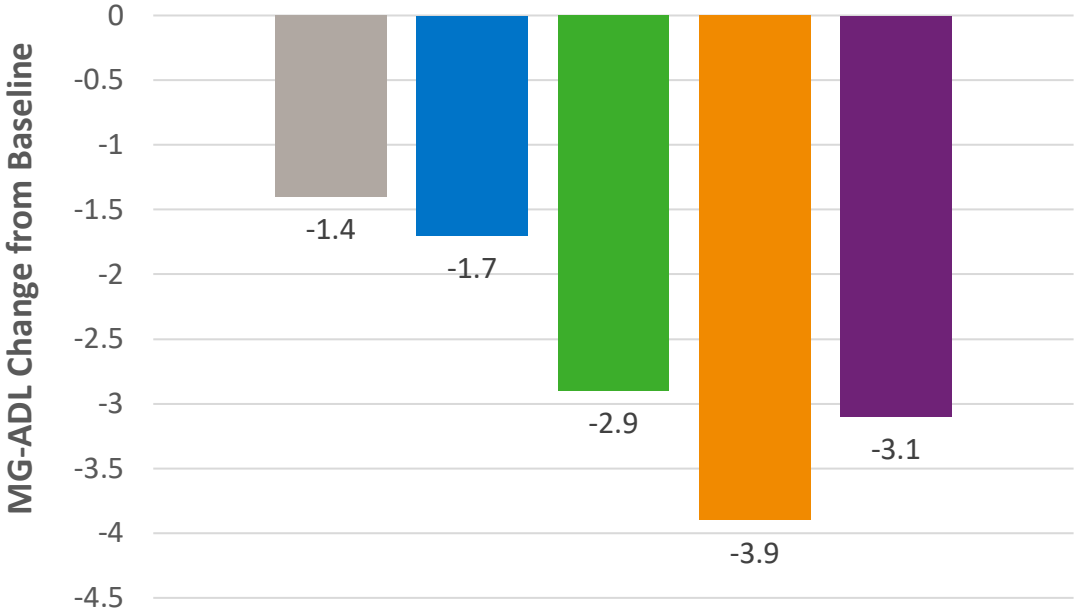


## Early Onset Responders (response within the first 2 weeks)

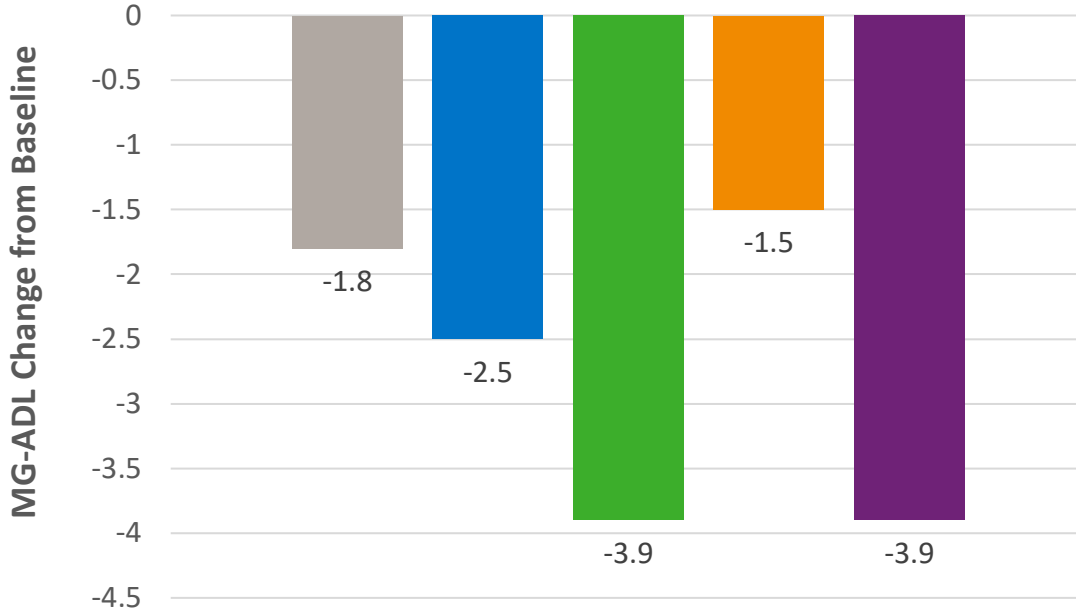


# Robust and Dose Responsive MG-ADL Improvement from Baseline

Day 29

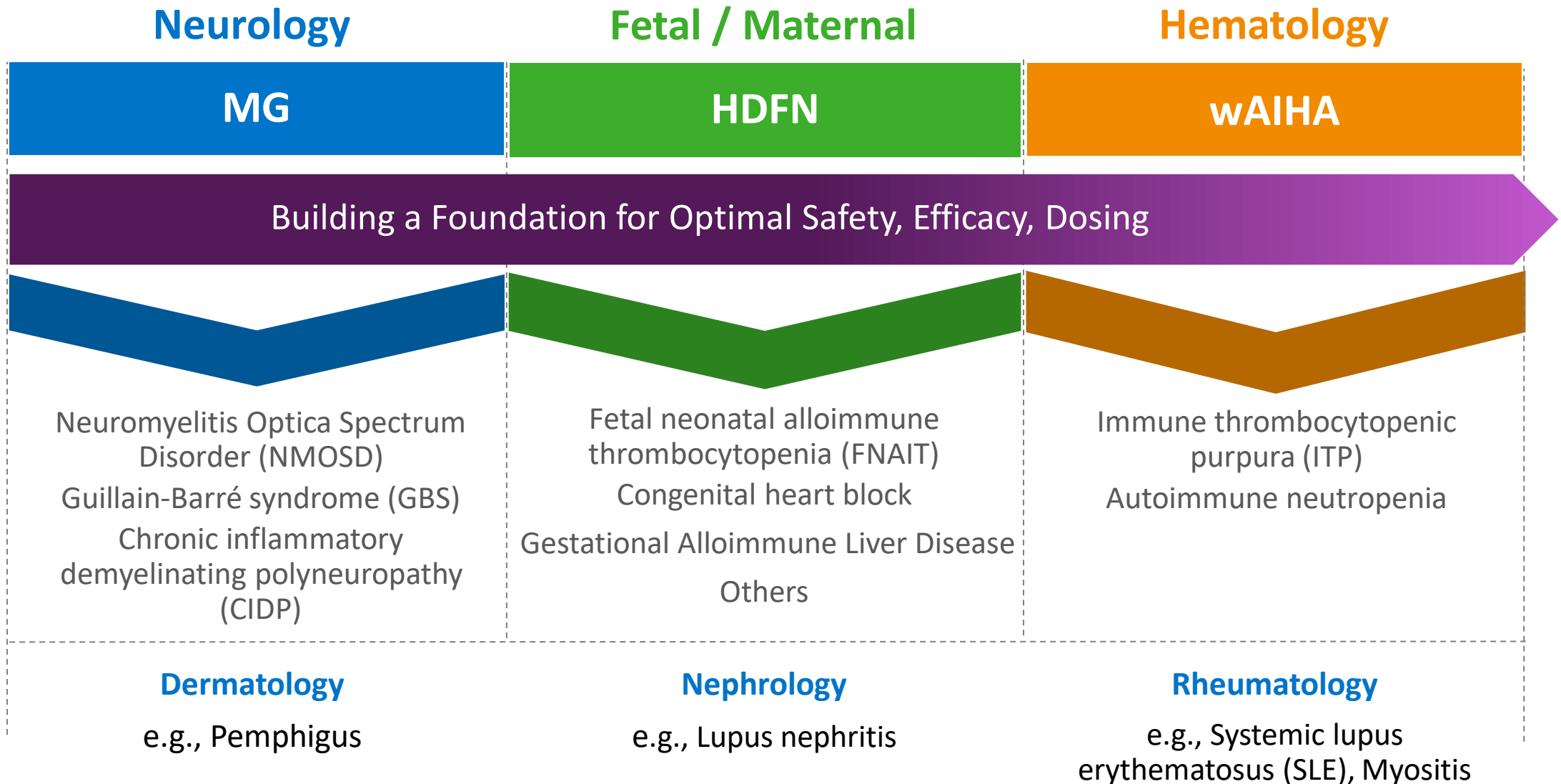


Day 57



Placebo    5 mg/kg Q4W    30 mg/kg Q4W    60 mg/kg single dose    60 mg/kg Q2W

# Building a Winning FcRn Franchise Based on Efficacy, Safety and Dosing





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