# Together We Discover

Reaching Patients Through Immunology Innovation

JP Morgan Healthcare Conference JANUARY 2021



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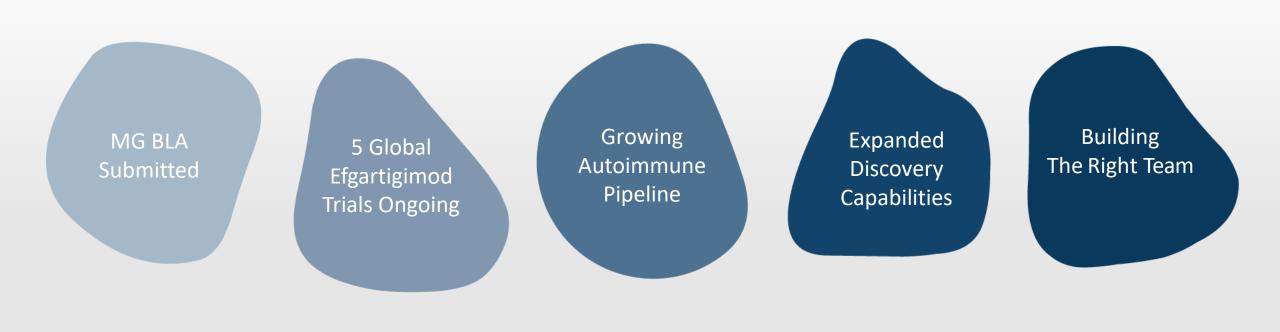
# Reaching People Living with MG in 2021



MORGAN, Living with MG



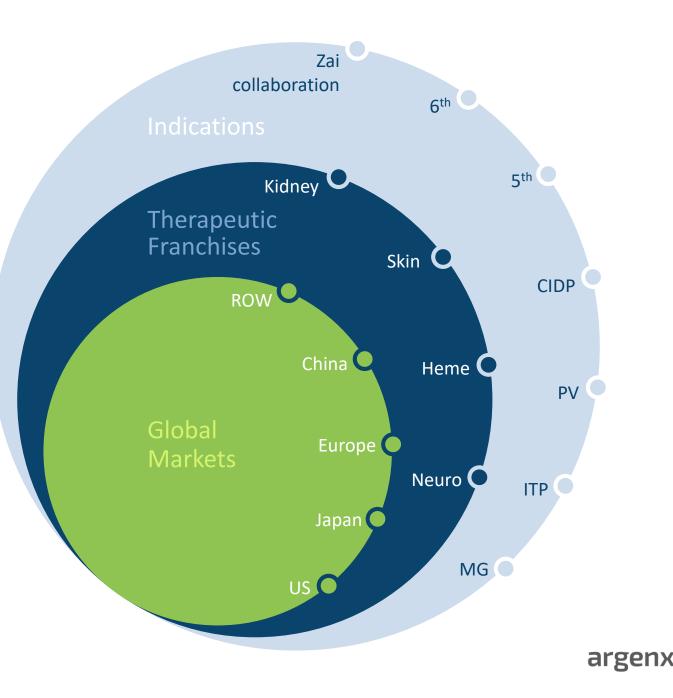
### 2020: Transformational Year for argenx





### Uniquely Positioned For exponential expansion

- $\circ$  efgartigimod indications
- $\circ$   $\,$  the rapeutic franchises
- $\circ$  global markets



**Reaching Patients Worldwide** 

# argenx + zai-ab

### Expand

patients we reach + number of indications

### Accelerate

enrollment into global registrational trials

### **Mutual Passion**

bringing innovative therapies to patients



### Deep Antibody Pipeline of Differentiated Candidates

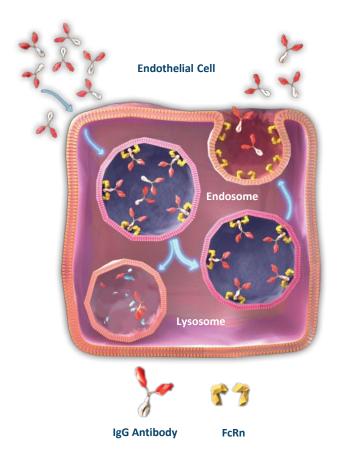
Program		Indication		Preclinical	Phase 1	Phase 2	Phase 3	Registration	Partner
	IV	MG							
	SC	MG							
	IV	ITP	advance						
	SC	ITP							
Efgartigimod	SC	PV	address						
	SC	CIDP	adhere v						
	SC	Fifth Indication							
	SC	Sixth Indication							
Cusatuzumab	+ AZA	Newly diag. AML (unfit)	CULMINATE				Janssen		
Cusatuzumab	+ AZA + VEN	Newly diag. AML (unfit)	ELEVATE						Janssen
ARGX-117	IV + SC	Autoimmune (MMN)							
ARGV-117	IV + SC	Kidney							
ARGX-118		Airway Inflammation							
ARGX-119		Neuromuscular indications							
ARGX-120		Undisclosed							

HEME

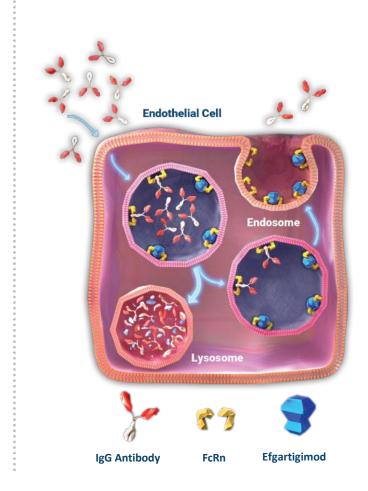


### FcRn Biology is Foundational to the Immune System

FcRn recycles IgG antibodies extending their abundancy



### Efgartigimod Blocks FcRn leading to IgG elimination



- Human IgG1 Fc fragment uniquely modulates FcRn, preserving characteristic pH dependent binding of endogenous IgG
- No impact on IgM, IgA or human serum albumin
- Does not affect IgG production, an important component to a vaccine response



### Efgartigimod: First-in-Class FcRn Antagonist

ERI Living with MG

- Proof-of-concept in three indications (MG, ITP, PV)
- IV and SC injection in development
- 350+ subjects or patients dosed
- Safety profile comparable to placebo in ADAPT trial

Patients on drug for more than 2 years

> CHRIS Living with MG

> > TERESA Living with MG

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# Patient Experience

"A person with MG on a good day operates at 70%. On bad days, you can get 10% out of your battery."

1/2 of Patients have been diagnosed with depression or anxiety in addition to gMG

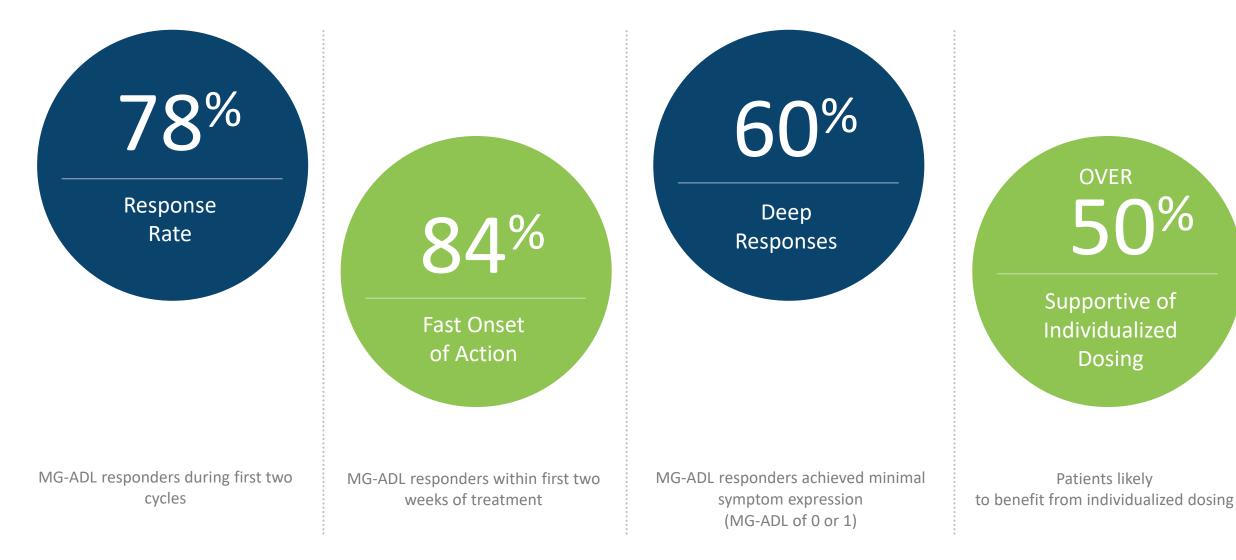
GLENN Living with MG Symptoms can vary from patient to patient, day to day, **or even throughout the same day**...this unpredictability contributes to emotional burden of disease 2.6 Years

mean time from symptom expression to diagnosis

- MG affects more than patients' muscles
- Surveyed neurologists ranked severe MG only behind ALS as most severe disease they treat



### Promising Value Proposition to MG Patients

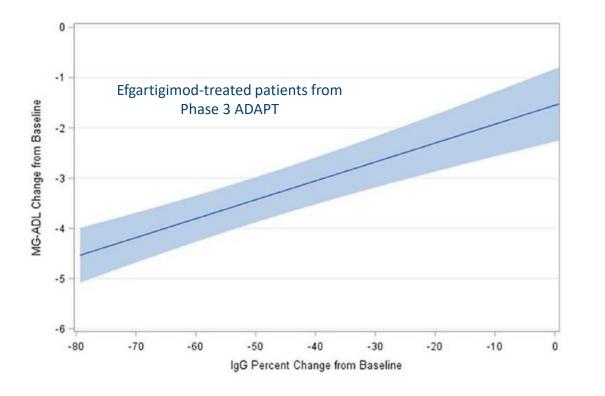


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# SC Bridging Strategy Leverages Correlation Between Pharmacodynamic and Clinical Effect



Established association of total IgG and MG-ADL following efgartigimod treatment



Bridging study (n=50) underway to support registration of SC efgartigimod

- Study designed to demonstrate noninferiority of PD effect of 1000 mg SC efgartigimod to 10mg/kg IV efgartigimod
- Phase 1 HV data showed 1000 mg SC efgartigimod has similar PD effect as 10mg/kg IV efgartigimod
- Additional patients from ADAPT+ to transition to SC efgartigimod

### Preparing for a Successful Launch



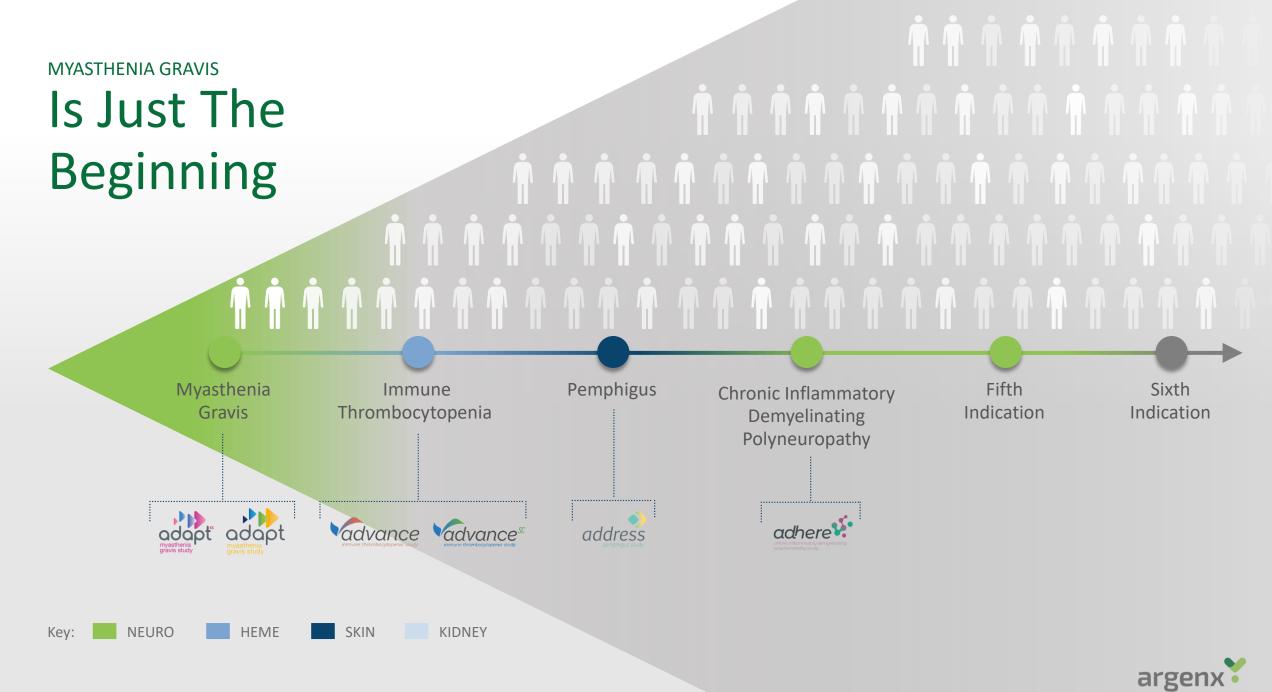
Listening to and Learning from MG Community

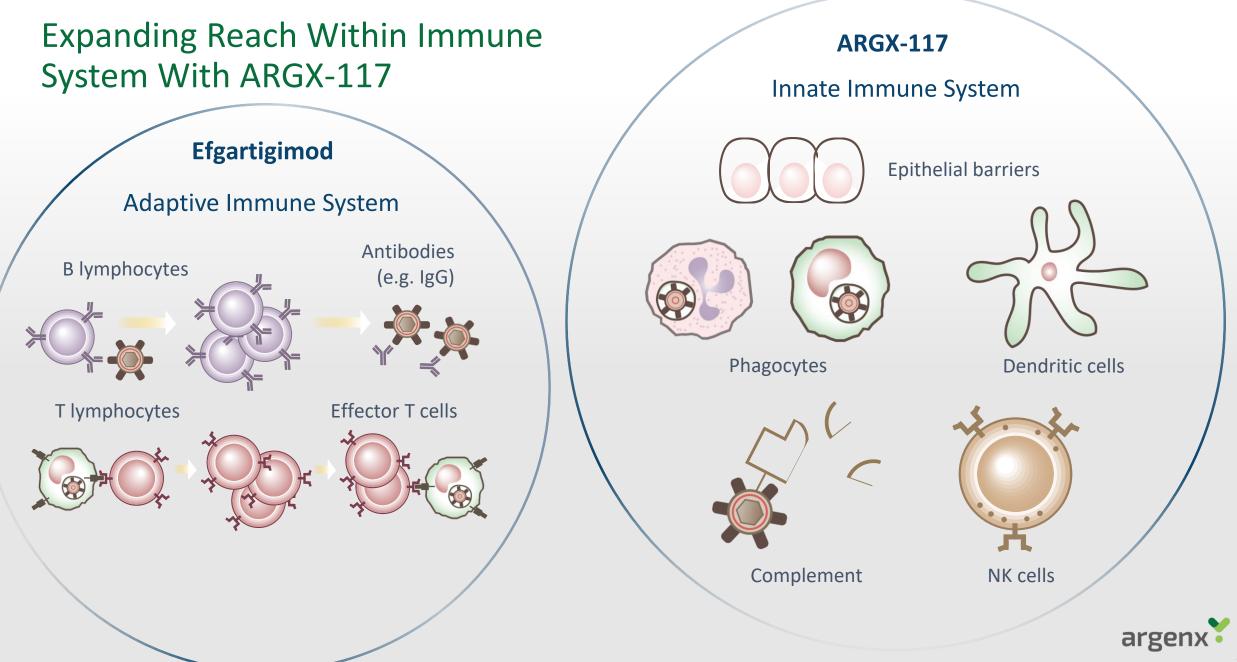
мgUnited

### A MYSTERY TO ME

### MyRealWorld<sup>™</sup> MG







### ARGX-117: Broad Opportunity By Targeting C2





### Cusatuzumab Strategy

Newly diagnosed elderly AML patients who are unfit for intensive chemotherapy

Phase 2 CULMINATE Trial Cusatuzumab + Azacitidine Go-forward dose selected	2	20 mg/kg
CR Rates	CR	CRc
	n=14	n=21
ITT (n=52)	27%	40%
Patients who received ≥ 2 cycles (n=33)	42%	64%
<b>30-day mortality: 5/52 (</b> CRc: CR, CRi, CRh	9.6%)	46.2% Adverse Risk Classification (ELN)

Phase 1b ELEVATE Trial in Triple Combination

cusatuzumab + azacitidine + venetoclax

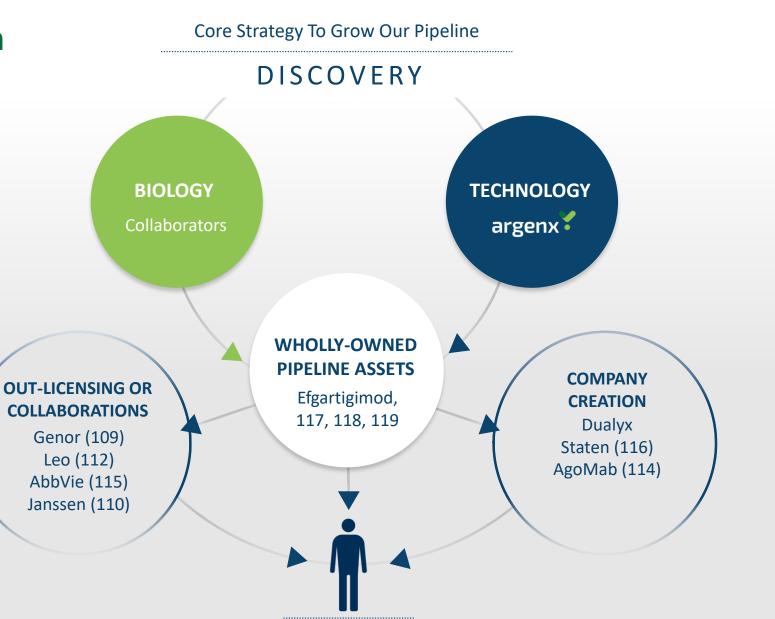
Decision to initiate additional studies will be determined following review of data from ELEVATE

**CRi:** Complete Remission with incomplete count recovery **CRh:** Complete Remission with partial recovery of peripheral blood counts

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### Immunology Innovation Program (IIP)

Optimizing the collision of great minds

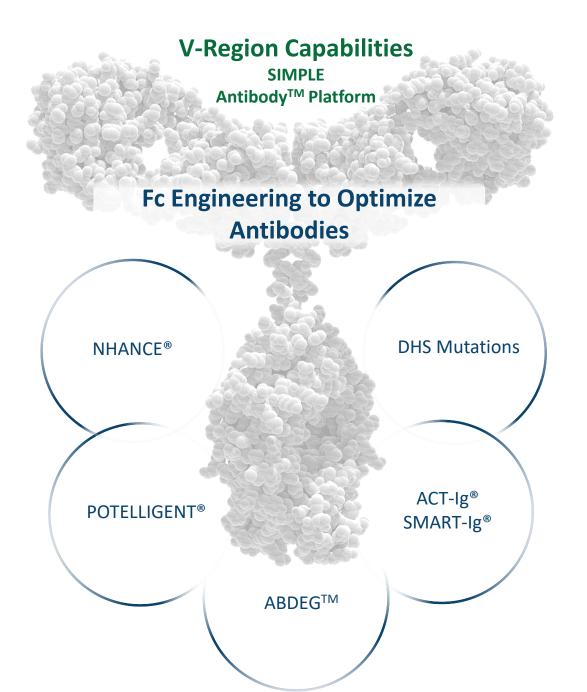


PATIENT



### Leading Antibody Discovery and Engineering Toolkit





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### Building Tomorrow's Immunology Company

Global expansion

# Strategic Priorities

Leverage IIP

Rooted in groundbreaking immunology research, growing through collaboration

Reach gMG patients with efgartigimod

Advance clinical development in multiple autoimmune indications

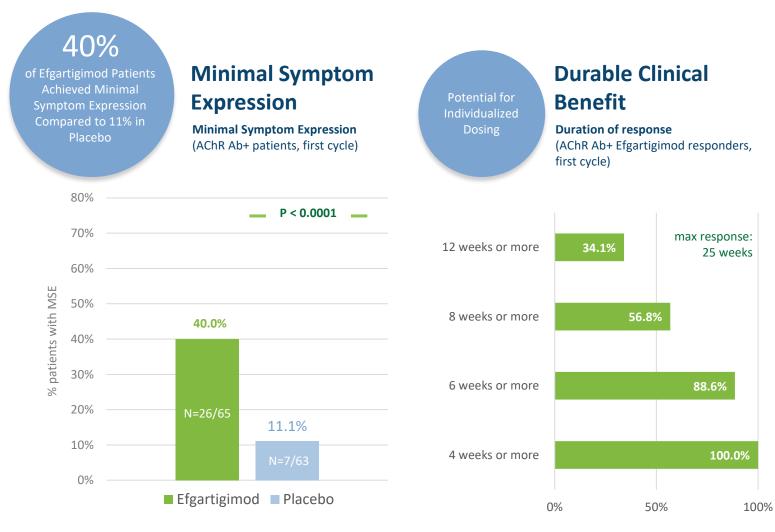
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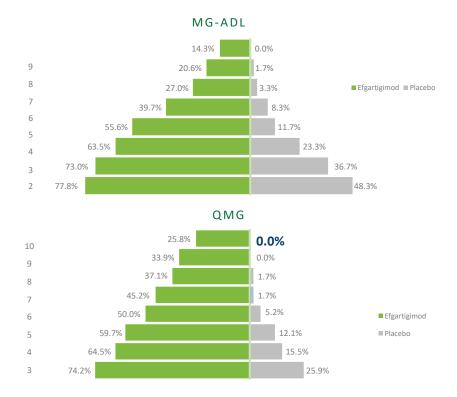


### ADAPT Data Showed Fast, Deep and Durable Responses



#### Efgartigimod Demonstrated Significant Magnitude Of Benefit

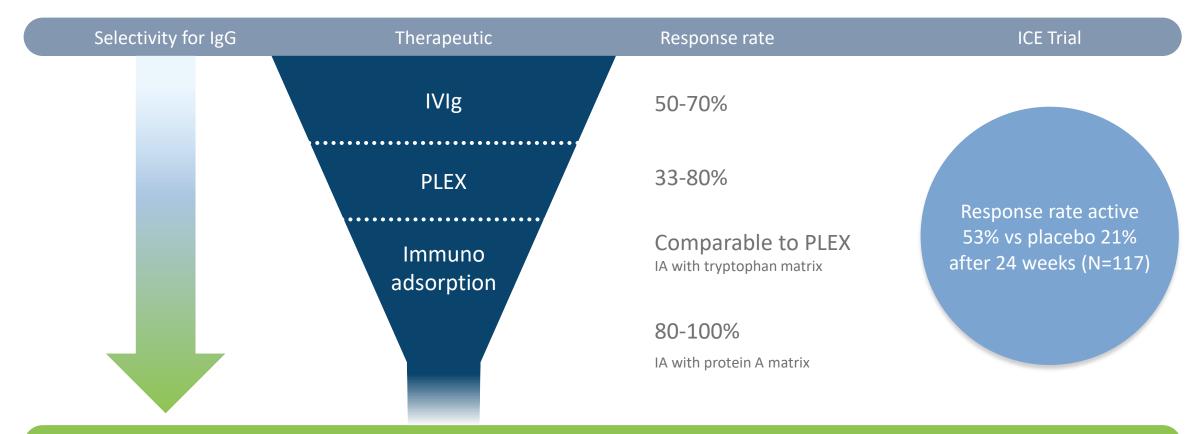
AChr-AB+ Patients, Cycle 1



**Minimal Symptom Expression:** MG-ADL = 0 (no symptoms) or 1 **Responder by Definition** is at Least 4 Consecutive Weeks

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### CIDP: Therapeutic Activity Shown With Increasing Selectivity For IgG Reductions



Clinical evidence for the role of pathogenic autoantibodies in CIDP



