



## JP Morgan Healthcare Conference January 2020 – San Francisco





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## argenx Today: Late-Stage Biotech Building Towards Commercial Success



New Data

PV

# argenx 2021: Reaching Patients

## Late-Stage Pipeline

A. M. MARILE

# **Therapeutic franchises**

# **Global expansion**



- ADAPT fully enrolled; data expected mid-2020
- 3/3 beachhead indications
- MyRealWorld<sup>™</sup> MG study

**Cusatuzumab strategic alliance** 

# Immunology Breakthroughs

Two new pipeline assets from IAP

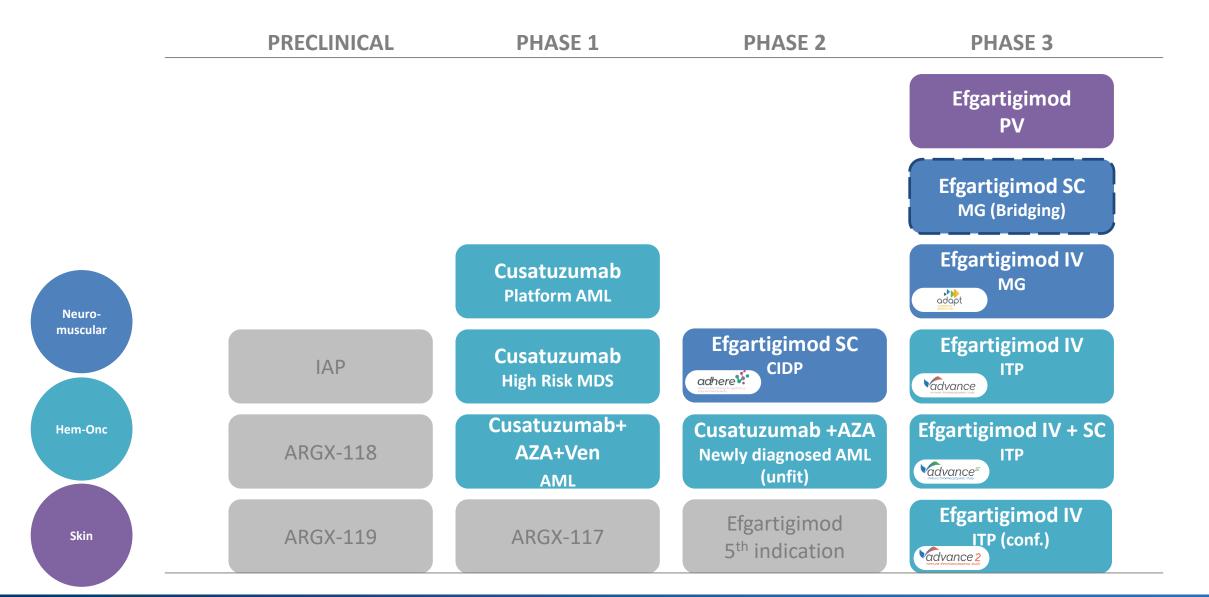
### argenx 2021: Growing Franchises With Multiple Late-Stage Programs





## View Of Pipeline: Poised To Have Five Phase 3 Trials Underway





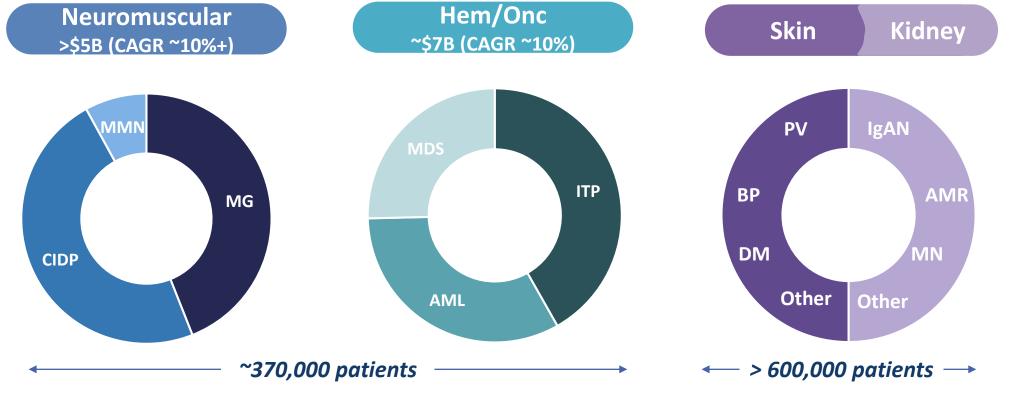
## **Building Deep Antibody Pipeline Of Differentiated Candidates**



PROGRAM	FIRST-IN-CLASS TARGET	INDICATION	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	BLA	MARKETED
Efgartigimod IV	FcRn	MG			-	Dat	a Mid-2020	
Efgartigimod SC Bridging	FcRn	MG					FDA Meeti	ng 2020
Efgartigimod IV	FcRn	ITP vadvance			Initia	ted 4Q19		
Efgartigimod IV + SC	FcRn					Initiate 2H20		
Efgartigimod IV	FcRn	ITP				Initiate 1H20		
Efgartigimod IV	FcRn	PV				Initiate 2H20		
Efgartigimod SC	FcRn	CIDP adhere		Initiate	d 4Q19 🔶	Go/No Go		
Efgartigimod	FcRn	5 <sup>th</sup> Indication			Announce in	2020		
Cusatuzumab + AZA	CD70	Newly diag. AML (unfit) CULMINATE			Da	ata 2020		
Cusatuzumab + AZA + VEN	CD70	Newly diag. AML (unfit)						
Cusatuzumab Platform	CD70	New AML settings and subpopulations		Initia	ate 1H20			
Cusatuzumab	CD70	Higher-risk MDS		Initia	ate 1H20			
ARGX-117	C2	Autoimmune including MMN		Initiate 1Q20				
ARGX-118	Galectin 10	Airway Inflammation						
ARGX-119	TBD	TBD		Announce 2020				6

## **Therapeutic Franchises Sit In High-Value Rapid-Growth Markets**





BP: Bullous pemphigoid DM: Dermatomyositis IgAN: IgA nephropathy AMR: Antibody-mediated rejection MN: Membranous nephropathy

## The Right Team In Place To Launch Efgartigimod





### **Preparing for Global Launch**

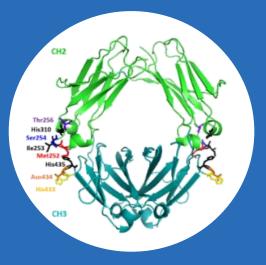






## **Building Differentiation Every Step Of The Way**









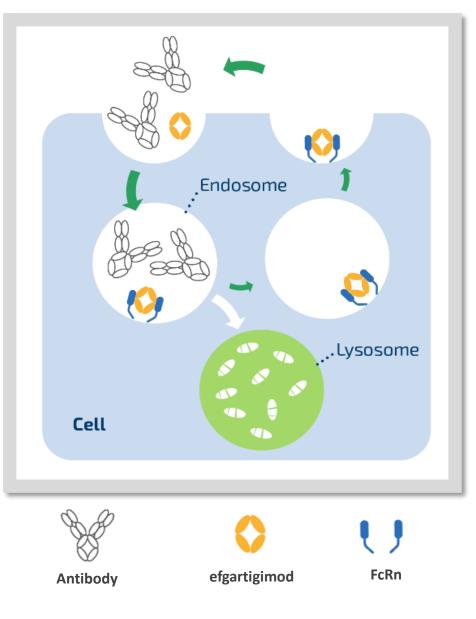
Molecule Design: Innovative Access Program **Clinical Development:** Thoughtful ADAPT Design

**Commercial Approach:** Real-world Evidence Study

## Efgartigimod: Unique Molecule Design Leads To Differentiated Profile in Phase 2



Efficacy 3/3 beachhead indications **Safety** No class effect

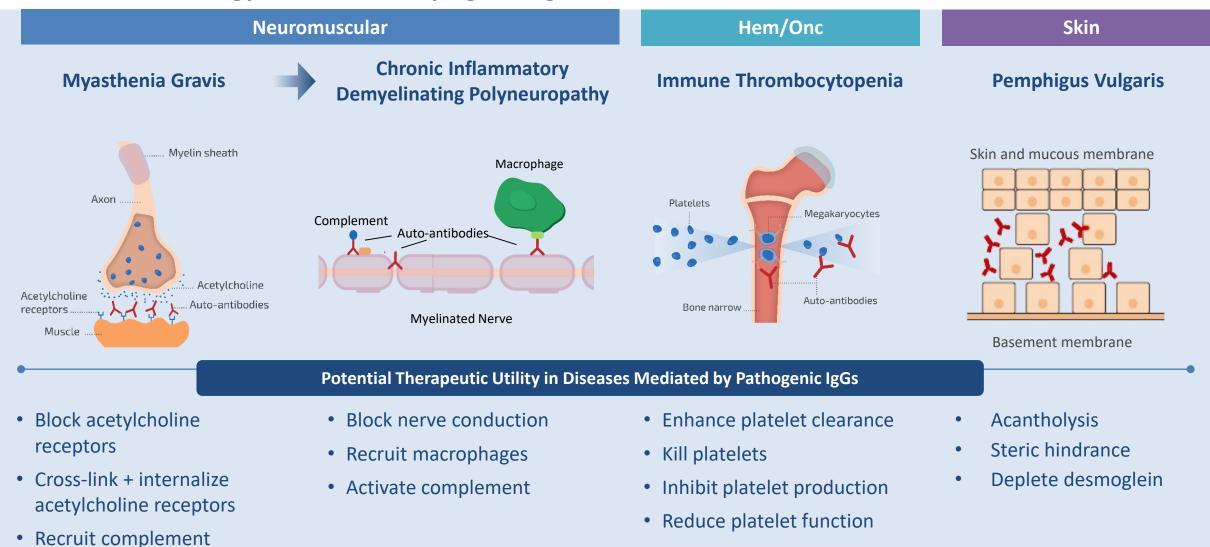


# Convenience

Potential optionality for patients



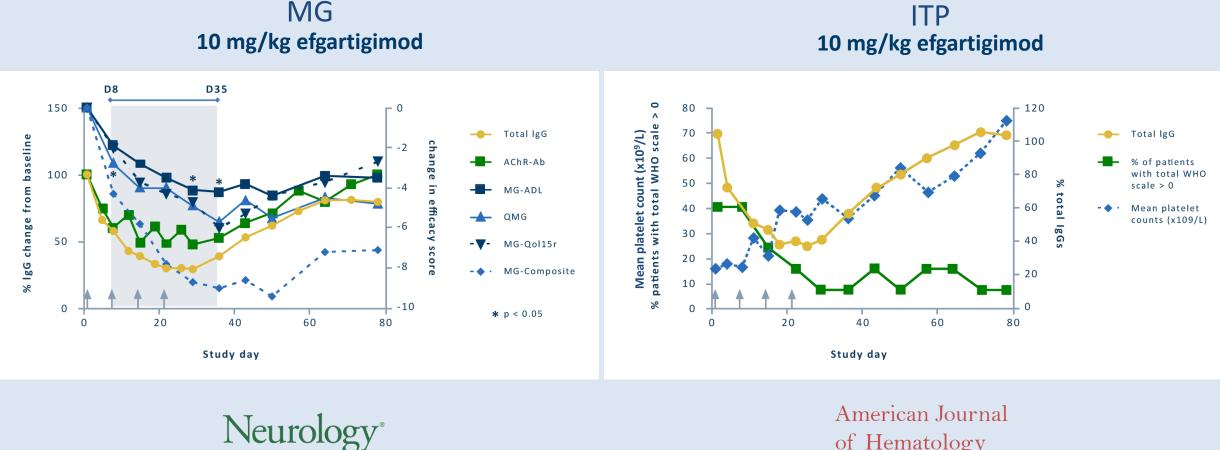
## **Beachhead Strategy Based On Unifying Biologic Rationale**



## Phase 2 Proof-Of-Concept Supports Advancement To Phase 3

IgG Reduction Correlates With Clinical Improvements





American Journal of Hematology

- Reduction of total and pathogenic IgGs led to clinically meaningful improvements in disease scores ٠ (MG-ADL, QMG, QoL and Composite for MG; platelet count and bleeding events for ITP)
- Favorable tolerability profile with adverse events balanced between active and placebo arms ٠

## Phase 2 Proof-of-Concept In PV Supports Advancement To Registrational Trial



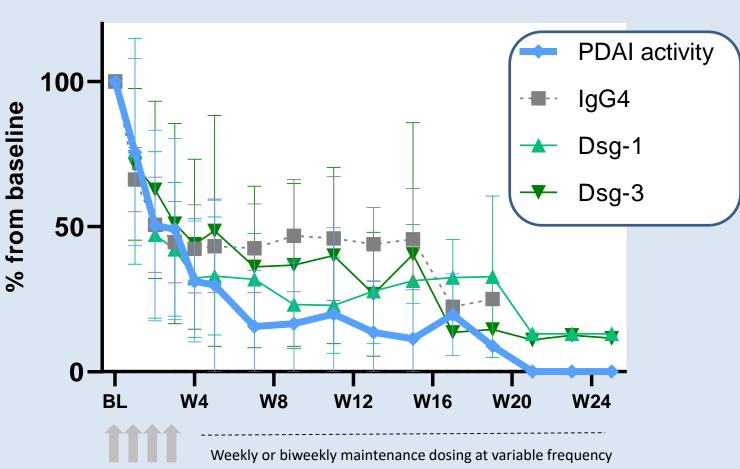
#### **Baseline Characteristics**

Pemphigus Vulgaris subtype Mucosal-dominant (N = 8) Mucocutaneous (N = 10) Cutaneous (N = 1) Pemphigus Foliaceus (N = 4)

**Severity** Mild: PDAI < 15 (N = 9) Moderate: PDAI 15-44 (N = 14)

> **Disease history** Newly diagnosed (N = 9) Relapsing (N = 14)





IgG Reduction Correlated to PDAI Score Improvement in Responders

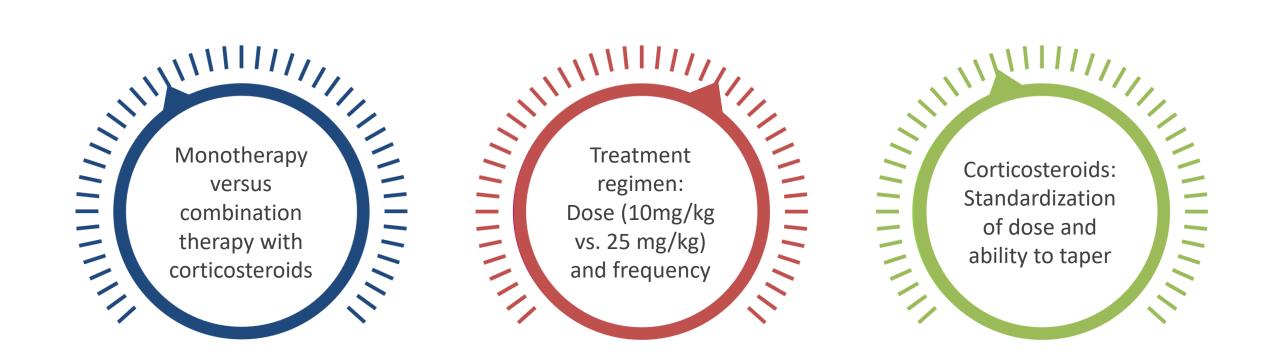
\* Eligible for efficacy analysis

Data cut off 7 Nov 2019; 8 patients on study at time of interim analysis

Data show efgartigimod treatment phases with at least biweekly dosing ; excludes IgG4 for one patient (outlier)

## **Efgartigimod In Pemphigus Vulgaris: Adaptive Phase 2 Trial**



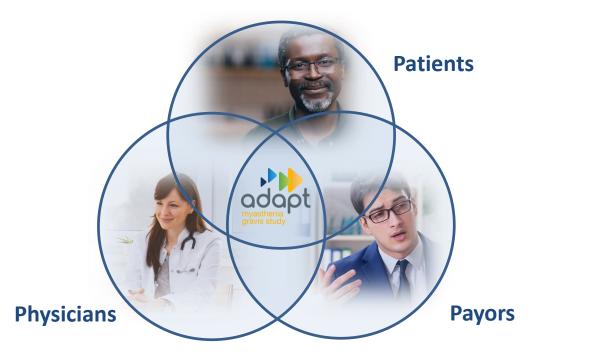




Fast onset of action	<b>78% disease control (18/23 patients) – majority after 1-2 infusions</b> Median time to DC: 14 to 15 days (mono/combo therapy)			
Deep responses	<b>70% clinical remission (5/7 patients) on optimized dosing regimen*</b> Time to CR: 2-10 weeks			
	Mean maximum PDAI improvement in responders >60% to >85% (mono/combo therapy)			
	Strong steroid sparing potential demonstrated			
Favorable tolerability	Determined by independent monitoring committee			
Potential synergy	Efgartigimod clears a-Dsg antibodies/Steroids stimulate Dsg synthesis			



### We listened to stakeholders...



Request to be tailored, convenient, cost-effective

### ...and built on observed attributes of efgartigimod

### Phase 2 MG data:

- **Fast onset of action**
- Responded within first four weeks

**Clinical response in 83% of patients** 

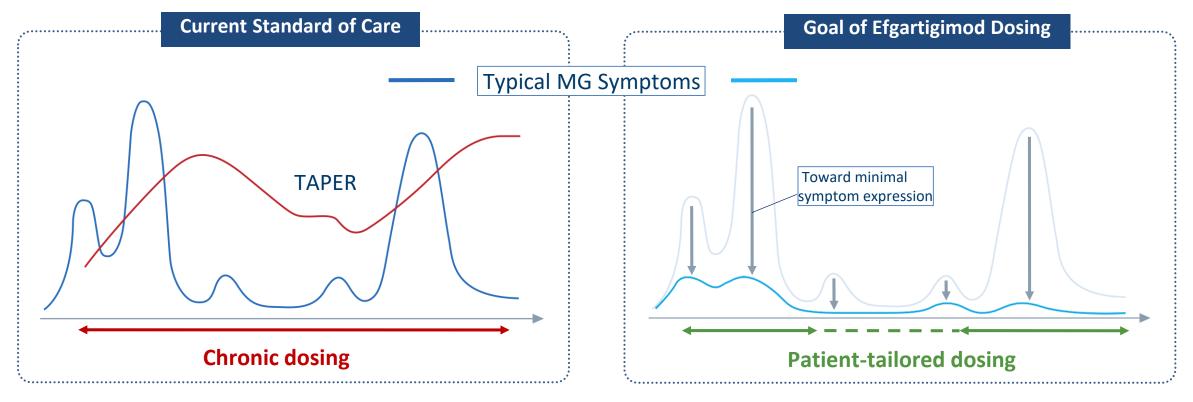
### **Durable response in 75% of patients**

• Sustained for at least 6 weeks

**Promising tolerability** 



# Efgartigimod Has Potential To Offer Tailored Treatment Approach In MG

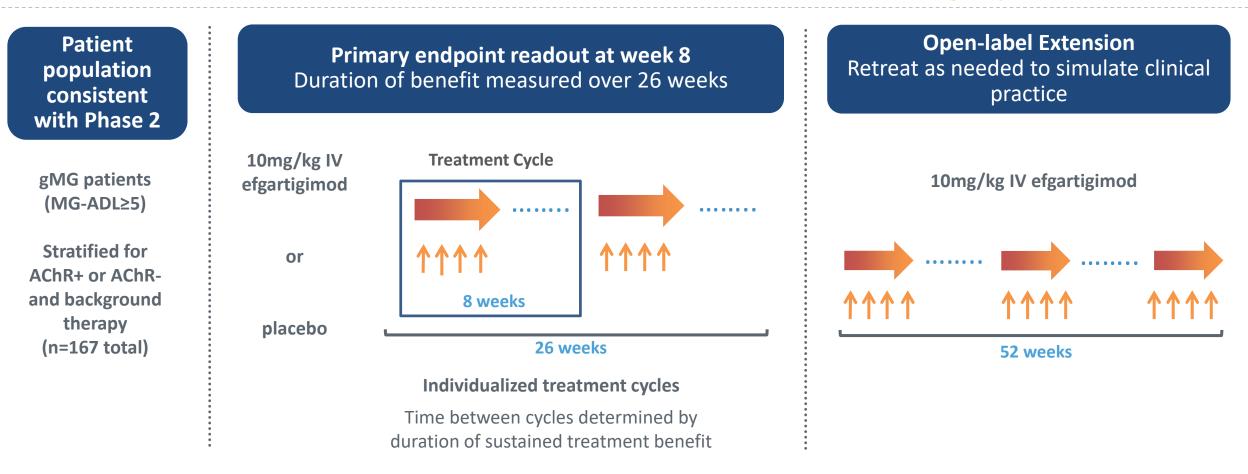


- Fast-acting steroids and slow-acting immunosuppressants
- Balancing symptom suppression and side effects

- Tailored regimen matches variability of MG
- Time between cycles is individualized
- Period of sustained therapeutic benefit between cycles can offer flexibility

## **Innovative ADAPT Design: Clinical Trial Designed To Meet Clinical Practice**





Primary endpoint (AChR+): % responders after first treatment cycle

Responder: ≥2 ADL points for at least 4 consecutive weeks <u>any time</u> within initial treatment cycle



## **Efgartigimod Has Potential To Disrupt Current MG Treatment Paradigm**

Vision: Efgartigimod positioned to be used early and more broadly within existing paradigm



<b>Current MG</b>	<b>Treatment Paradigm</b>
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ACIs (mestinon) at diagnosis Steroids most common add-on ISTs used for steroid sparing

Later agents used for severe/refractory/crisis



## First of its kind in MG





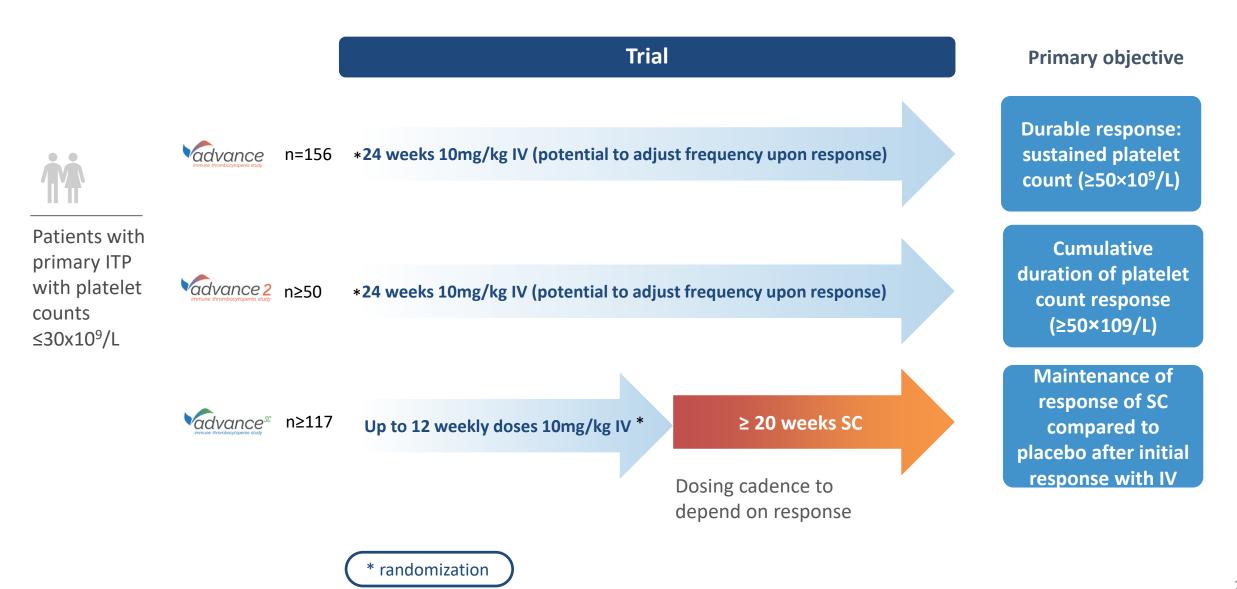


Global prospective – longitudinal - observational

Voice of ≥2000 patients - digitally

Patient perspective on diagnosis, treatment, symptom, economic and humanistic burden





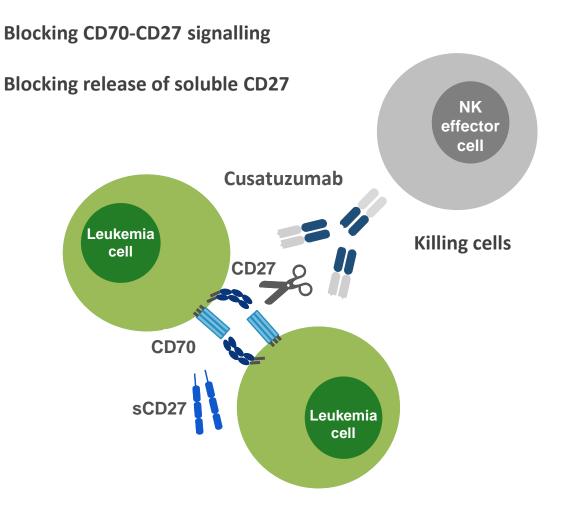




Identify patients with active CIDP		Confirm IgG autoantibody involvement	Document efficacy & safety efgartigimod vs placebo	
		Treatme		
		Open-label	Placebo-controlled	
Screening	Run-in period	Stage A	Stage B (stage A responders only)	Efficacy analysis
Confirmation of diagnosis by independent committee	<ul> <li>Worsening of disease within 12 weeks after drug withdrawal (INCAT, I-RODS, grip strength)</li> </ul>		Placebo weekly SC	based on relapse (adjusted INCAT) Study endpoint with 88 relapse events in stage B
	<ul> <li>Newly diagnosed/treatment naïve skip run-in period</li> </ul>	Efgartigimod weekly SC	Efgartigimod weekly SC	N=sample size estimation ~120-130 Followed by
≤4weeks	≤13weeks	Up to 12 weeks, until clinical improvement (ECI)	Up to 48 weeks	Open Label Extension study

Go/No Go N=30





Joint development plan focused on AML, MDS and other heme malignancies

Upfront \$300M + \$200M equity @ 20% premium, up to \$1.3B in milestones, double digit royalties OUS

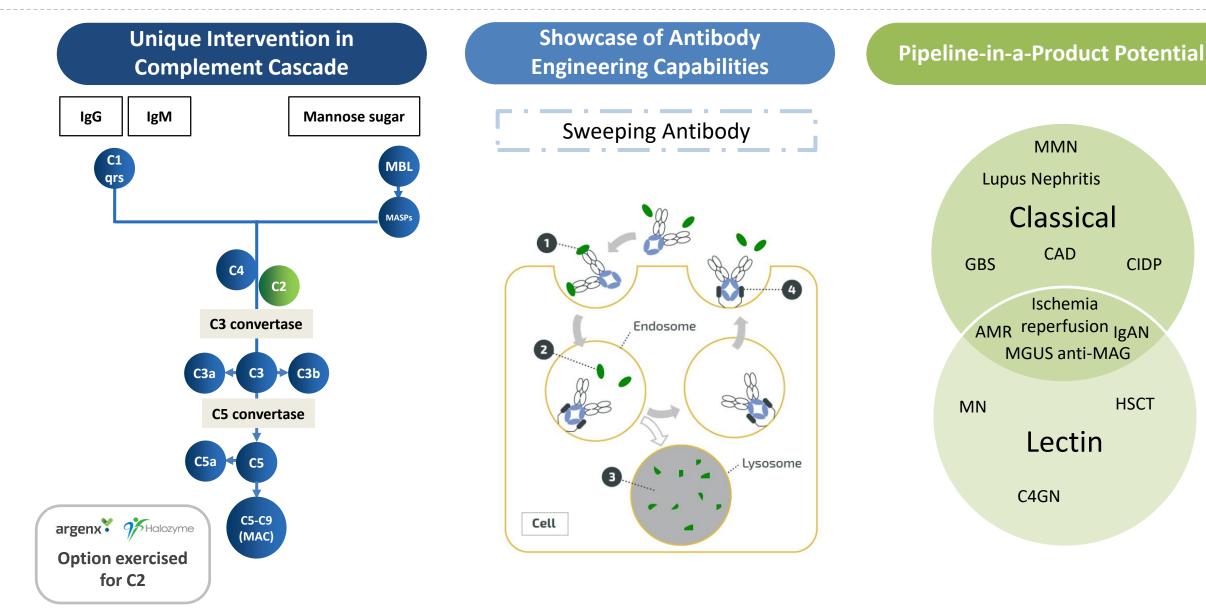
50% of US economics on a royalty basis, up to 50% commercial efforts

- First two trials underway on time and as planned
- Additional trials to start in 2020 in AML settings and subpopulations, and MDS

Achieved first milestone payment under collaboration for enrollment progress in CULMINATE



### **ARGX-117: Sweeping Antibody Targeting C2**





Accessing First-in-Class Targets by Collaborating with Leading Research Biologists

#### argenx

Antibody Expertise SIMPLE Antibody™, NHance®, ABDEG™, POTELLIGENT®

### Academic Institutions & Biotechs

## **Disease Biology Expertise**

Texas A&M, Bern, Utrecht, Louvain, Penn, Columbia, Torino, de Duve, VIB

## Co-creating immunology solutions: building beyond each individual contribution



8 assets from Innovative Access Program have delivered value to argenx









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