

Efgartigimod Phase 2 in Immune Thrombocytopenia: Topline Data



17 September 2018

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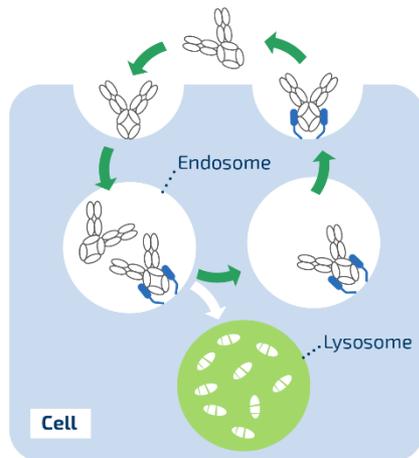


**Efgartigimod:
A Pipeline-in-a-Product Opportunity**

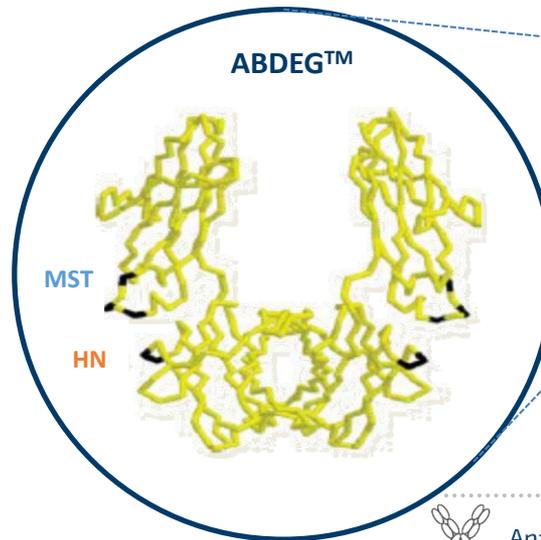
Efgartigimod Exploits Natural Fc/FcRn Interaction Site

Leveraging Proprietary ABDEG™ Technology

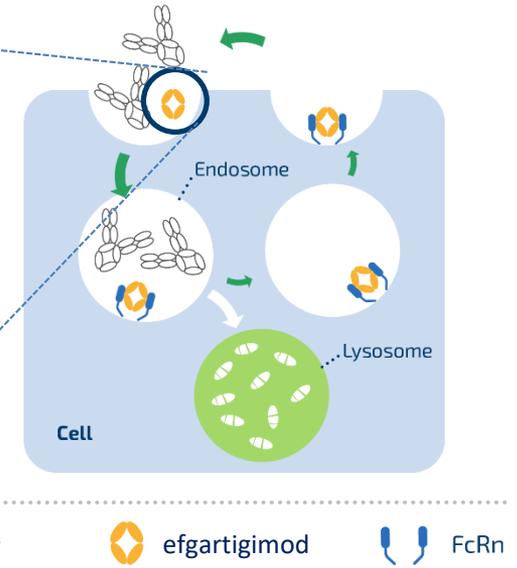
IgG antibodies recycle through FcRn⁽¹⁾...



efgartigimod potentially blocks FcRn...



leading to IgG elimination



- Human IgG1 Fc-fragment that utilizes ABDEG™ Fc engineering technology⁽²⁾⁽³⁾⁽⁴⁾
- Targets and binds to FcRn blocking recycling of IgG leading to elimination of IgG antibodies⁽³⁾⁽⁴⁾
- Cannot engage Fcγ receptors when bound to its target FcRn⁽³⁾⁽⁴⁾
- Pathogenic IgG antibodies mediate multiple autoimmune diseases

(1) Roopenian et al. 2007, Nat Rev Immunol.

(2) Vaccaro et al. 2005, Nat Biotech.

(3) Ulrichs et al. 2018, JCI

(4) argenx data



Primary Adult Immune Thrombocytopenia (ITP) – a Severe Autoimmune Disorder

What is ITP?

- Rare autoimmune bleeding disease
 - Estimated 69,300⁽¹⁾ patients in US
 - ~80% diagnosed with primary ITP
 - Newly diagnosed: ~3,000 – 7,500 patients ⁽¹⁾
 - Persistent: ~4,500 patients⁽²⁾
 - Chronic: ~43,000 patients⁽²⁾
- Symptoms include: mild bruising to severe bleeding, fatigue, fear of bleeding, impact on work and social activities, depression
- Relevance of platelet counts
 - $\leq 30 \times 10^9/L$ generally accepted trigger for therapy
 - Improvement to $\geq 50 \times 10^9/L$ considered clinically meaningful

Limited treatment options

- Multiple iterations on corticosteroids & IVIg
- TPO-receptor agonists*
- Splenectomy
- Immunomodulatory agents

* Generated global revenues of \$1.5 billion in 2017⁽³⁾⁽⁴⁾

Unmet need in ITP

- Current treatments – limited efficacy and significant side effects
- No real treatment paradigm exists – trial & error
- Patients adapt life style to cope with disease burden and treatment side effects

(1) Fogarty et al. 2004 Hematol Oncol Clin North Am, Feudjo-Tepie et al. 2008. J. Thromb Haemost, Segal et al. 2004, Am. J. of Hematol.

(2) Extrapolated from Moulis et al. 2017, Am. J. Hematol.

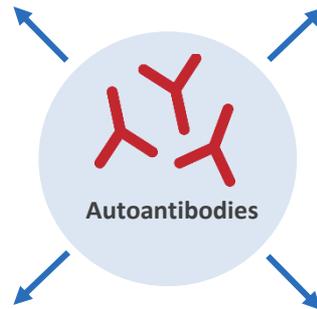
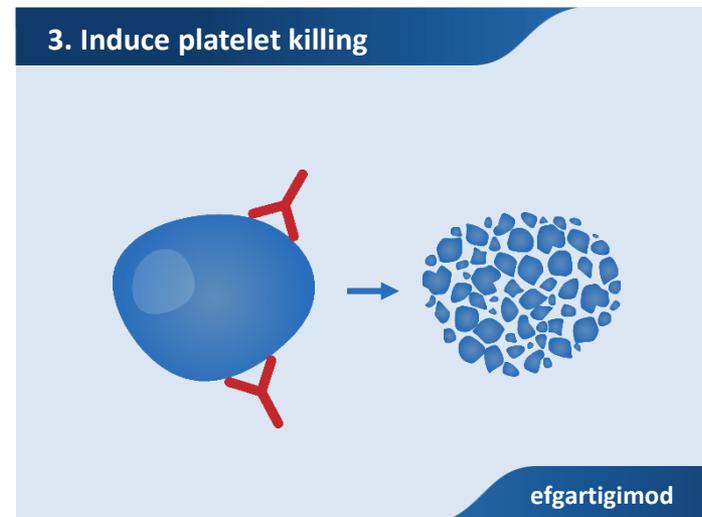
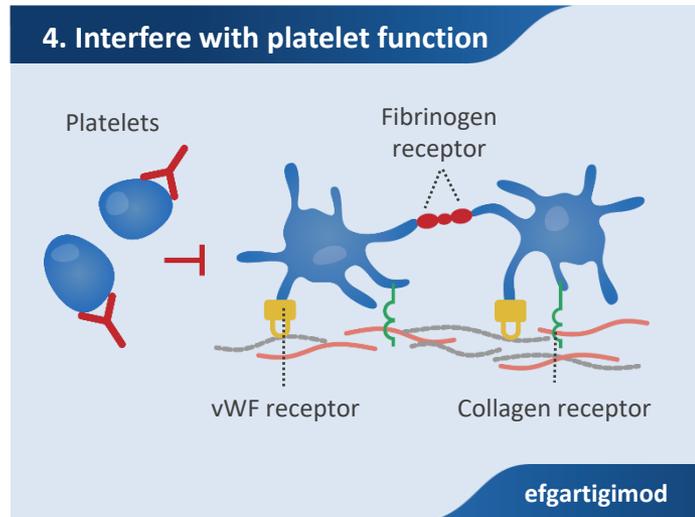
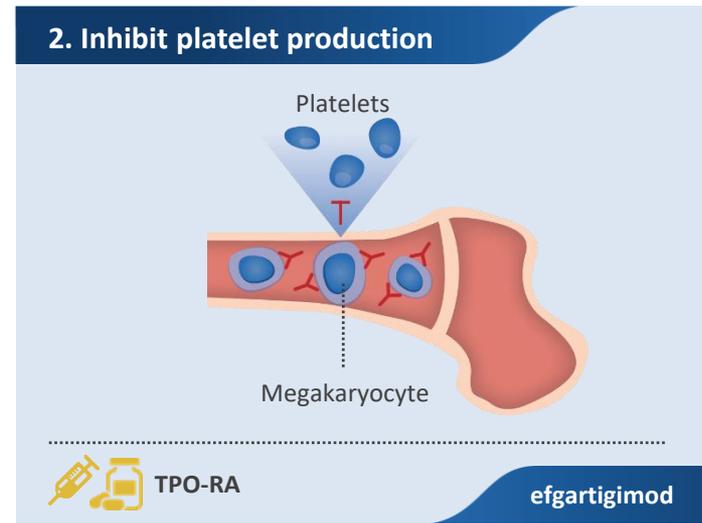
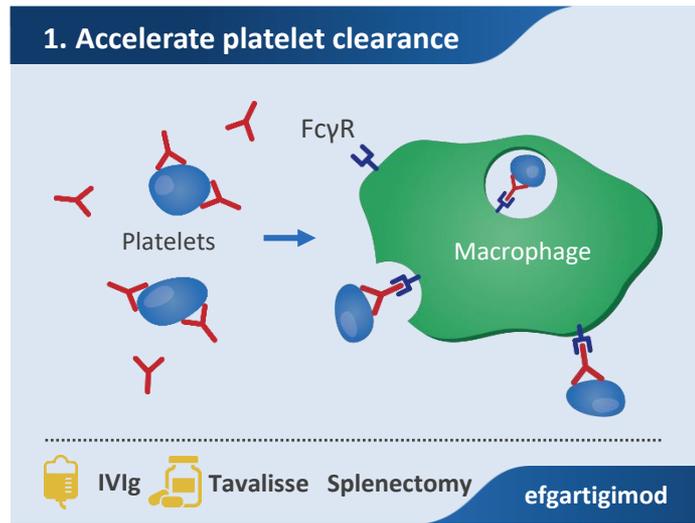
(3) Novartis FY 2017

(4) Amgen FY 2017

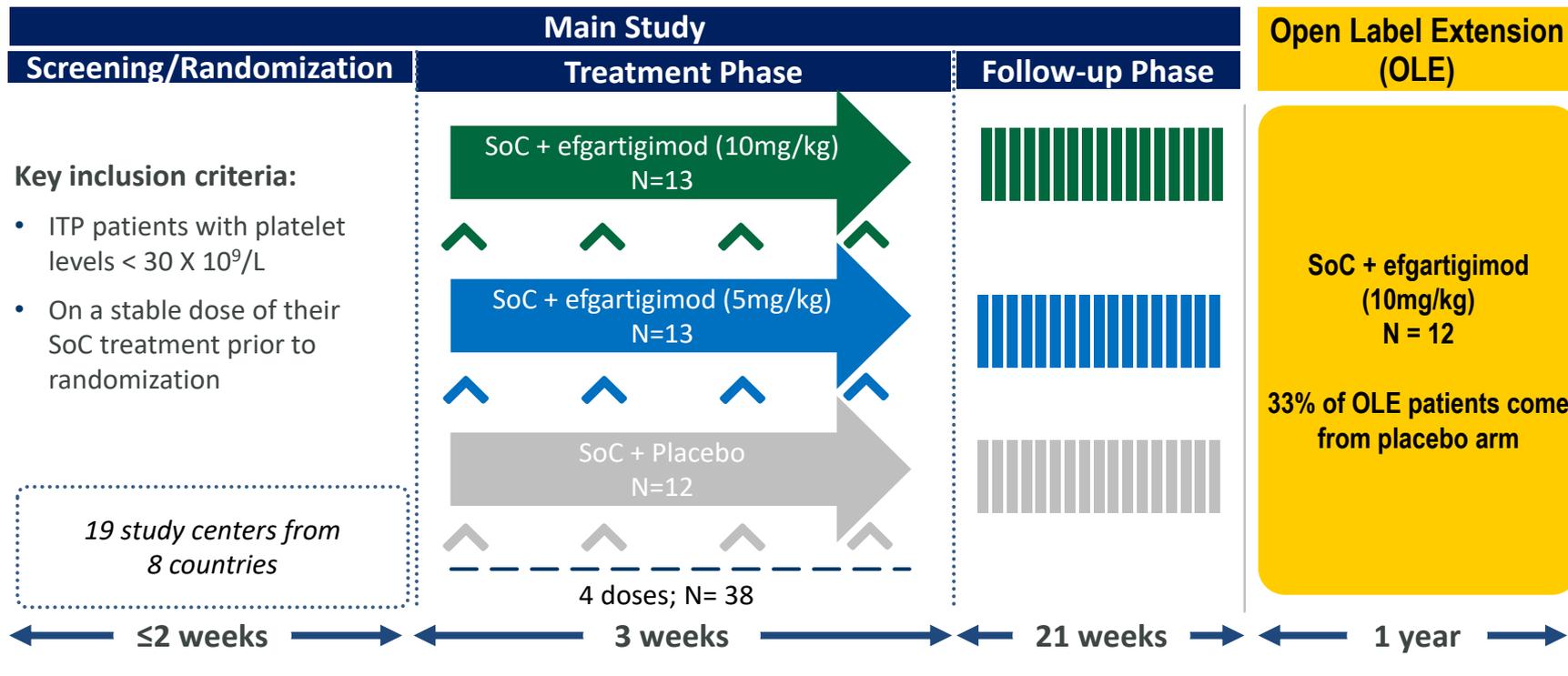


Efgartigimod Targets All Pathogenic AutoAb Actions Simultaneously

Potential to eliminate therapeutic cycling based on trial-and-error



ITP Amended Phase 2 Trial Design



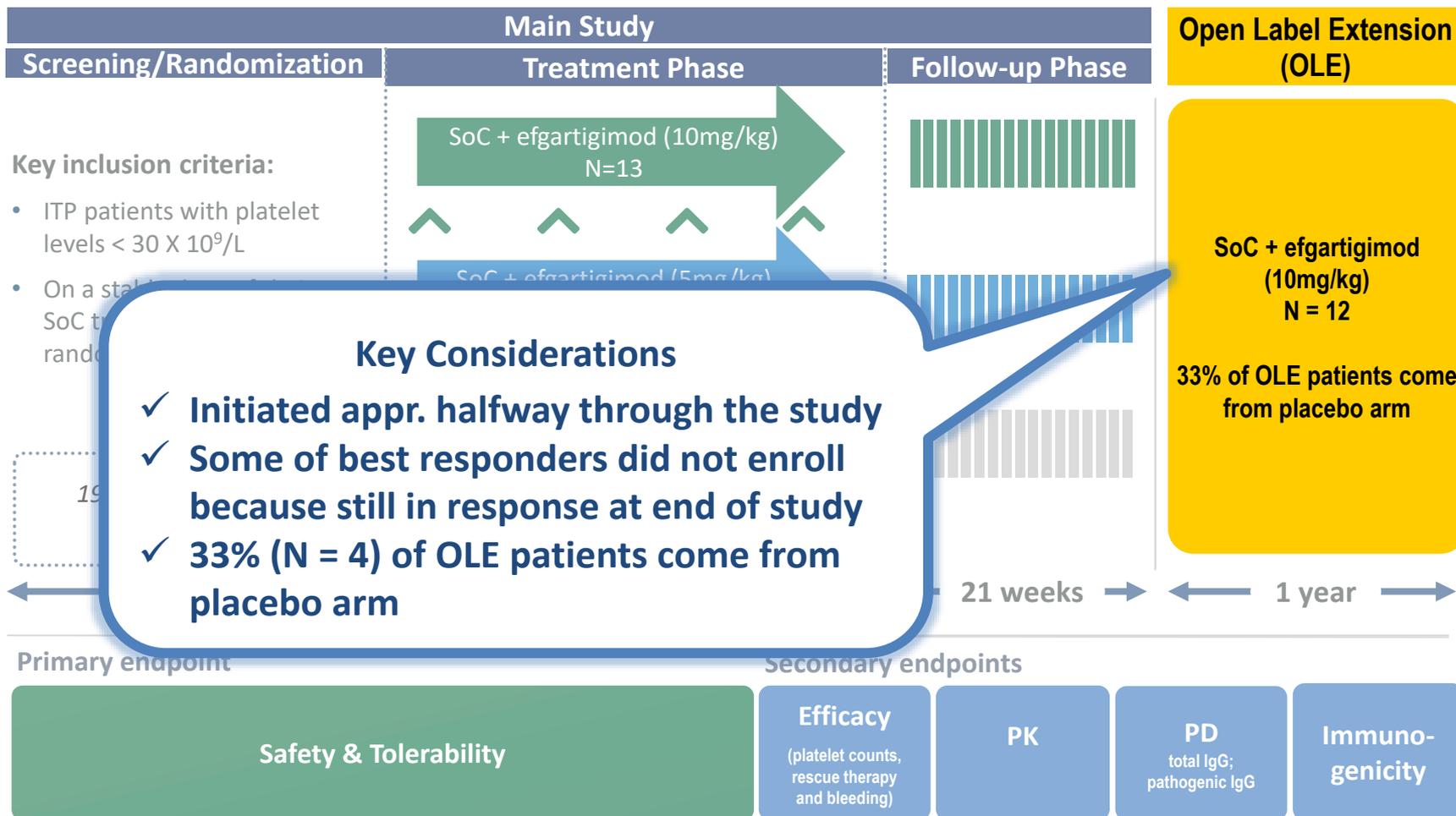
Primary endpoint

Safety & Tolerability

Secondary endpoints

- Efficacy**
(platelet counts, rescue therapy and bleeding)
- PK**
- PD**
total IgG; pathogenic IgG
- Immuno-genicity**

ITP Amended Phase 2 Trial Design



Baseline Population and Disease Characteristics

	Placebo (N = 12)*	Efgartigimod: 5mg/kg (N = 13)	Efgartigimod: 10 mg/kg (N = 13)**
Age, median, (range)	38.5 (19 - 69)	41.0 (22 - 77)	46.0 (29 - 62)
Gender, N (%)			
• Male	5 (41.7)	4 (30.8)	9 (69.2)
• Female	7 (58.3)	9 (69.2)	4 (30.8)
Race, N (%)			
• White	11 (91.7)	12 (92.3)	13 (100)
• Not reported	1 (8.3)	1 (7.7)	-
ITP Classification, N (%)			
• Newly diagnosed (≤ 3 months)	-	2 (15.4)	-
• Persistent (> 3 and < 12 months)	3 (25.0)	1 (7.7)	4 (30.8)
• Chronic (≥ 12 months)	9 (75.0)	10 (76.9)	9 (69.2)
Duration of ITP, median (range), years	3.5 (0.3 - 47.8)	4.5 (0.1 - 34.2)	5.4 (0.7 - 28.7)
Baseline platelet count, mean, / μ L (range)	18 (4 - 40)	18 (6 - 49)	15 (5 - 35)
Baseline platelet count of $< 15k/\mu$ L, N (%)	6 (50.0)	7 (53.8%)	7 (53.8)
SoC at baseline			
• Corticosteroids N (%)	3 (25.0)	10 (76.9)	6 (46.2)
• TPOs N (%)	3 (25.0)	4 (30.8)	3 (23.1)
• Immunosuppressants N (%)	1 (8.3)	-	1 (7.7)
• Watch & Wait N (%)	4 (33.3)	2 (15.4)	5 (38.5)
• Other N (%)	1 (8.3)	1 (7.7)	-



• Four placebo patients were discontinued before the end of the main study ** Two 10mg/kg patients were discontinued before receiving all 4 infusions argenx data

Favorable Tolerability Profile

Consistent with efgartigimod clinical studies to date

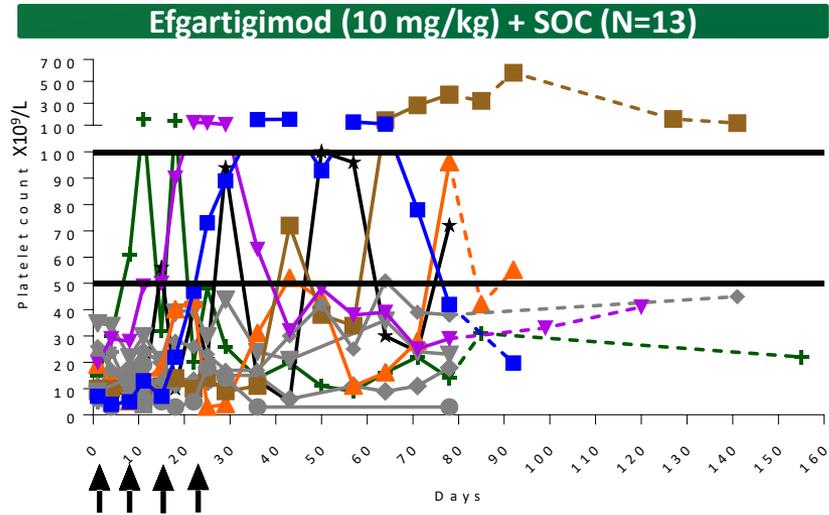
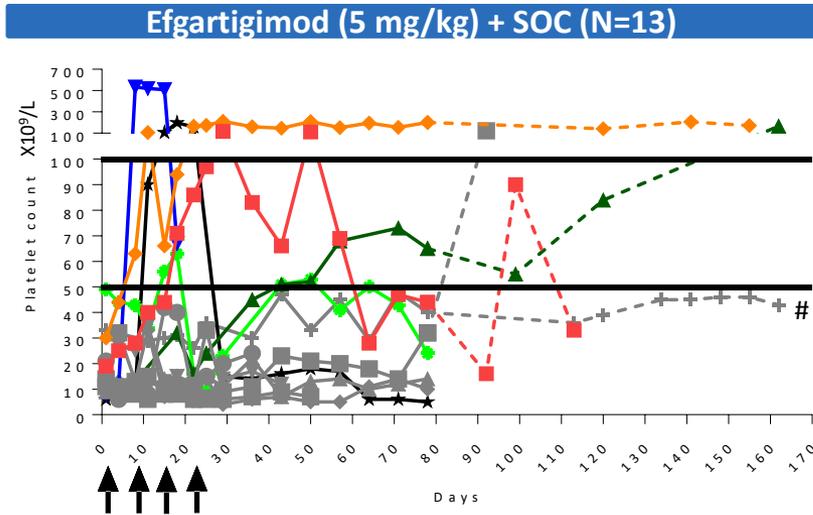
- Well-tolerated profile: consistent with Phase 2 MG and Phase 1 healthy volunteer trials
- TEAEs profile balanced between efgartigimod and placebo arms
- TEAEs mostly mild (grade 1) in severity; one non-study drug related SAE (viral infection)
- No deaths or TEAEs leading to discontinuation of treatment reported

Treatment Emergent Adverse Events (TEAEs) Reported in ≥ 2 subjects (non-bleeding)	Placebo (N = 12)	Efgartigimod 5 mg/kg (N = 13)	Efgartigimod 10 mg/kg (N = 13)
Most common TEAEs N (%)			
• Headache	2 (16.7)	1 (7.7)	-
• Hypertension	1 (8.3)	-	2 (15.4)
• Vomiting	-	-	2 (15.4)
• Cystitis	-	1 (7.7)	1 (7.7)
• Rash	-	1 (7.7)	1 (7.7)
• Productive cough	1 (8.3)	1 (7.7)	-
TEAEs <i>deemed related</i> to study intervention N (%)			
• Headache	1 (8.3)	-	-
• Vomiting	-	-	1 (7.7)
• Pubic pain	1 (8.3)	-	-
• Vaginal discharge	1 (8.3)	-	-
• Amenorrhoea	1 (8.3)	-	-

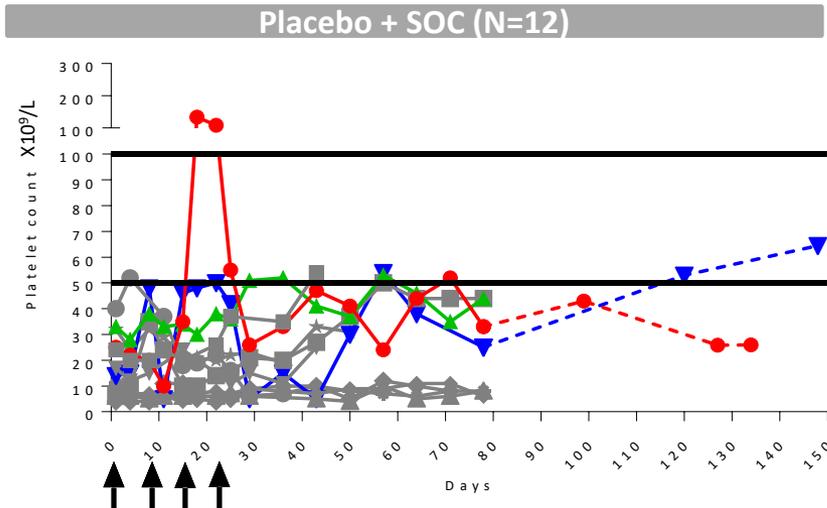
Clinically Meaningful Improvements in Platelet Counts

Effect demonstrated across newly diagnosed, persistent and chronic forms of ITP

Efgartigimod



Placebo

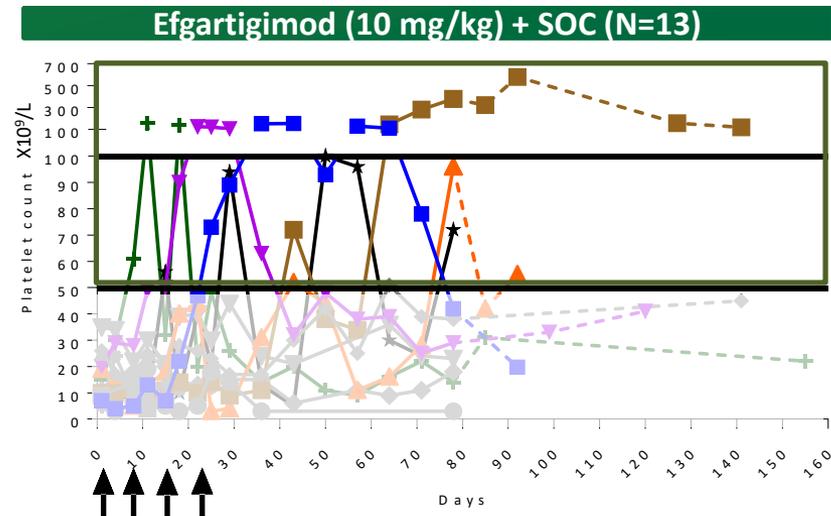
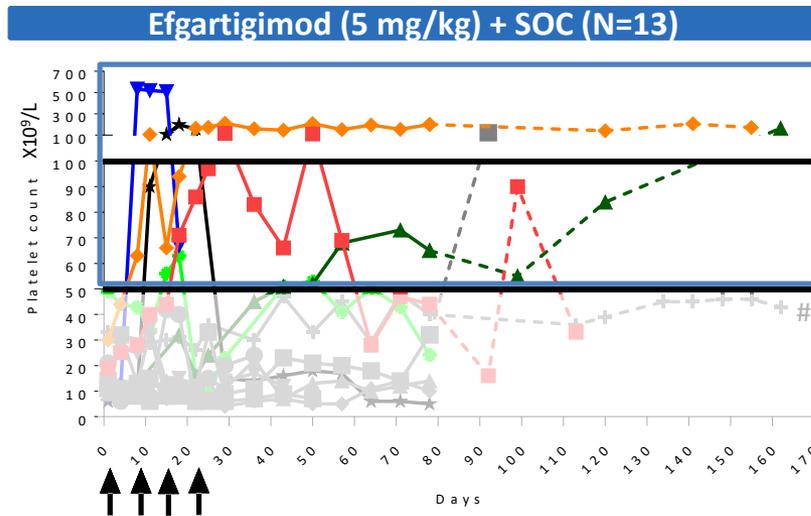


Color: patients achieving $\geq 50 \times 10^9/L$, at least two visits. Note: All central lab values for the main study, except for patient marked by (#). All local lab values for the extended follow-up > 78d. Extended follow-up period shown by dotted lines, note frequency of visits dependent on medical need in this period

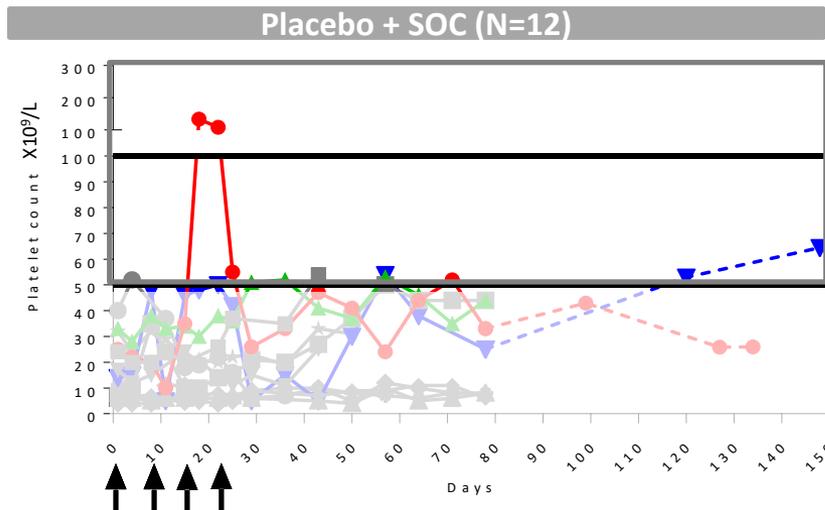
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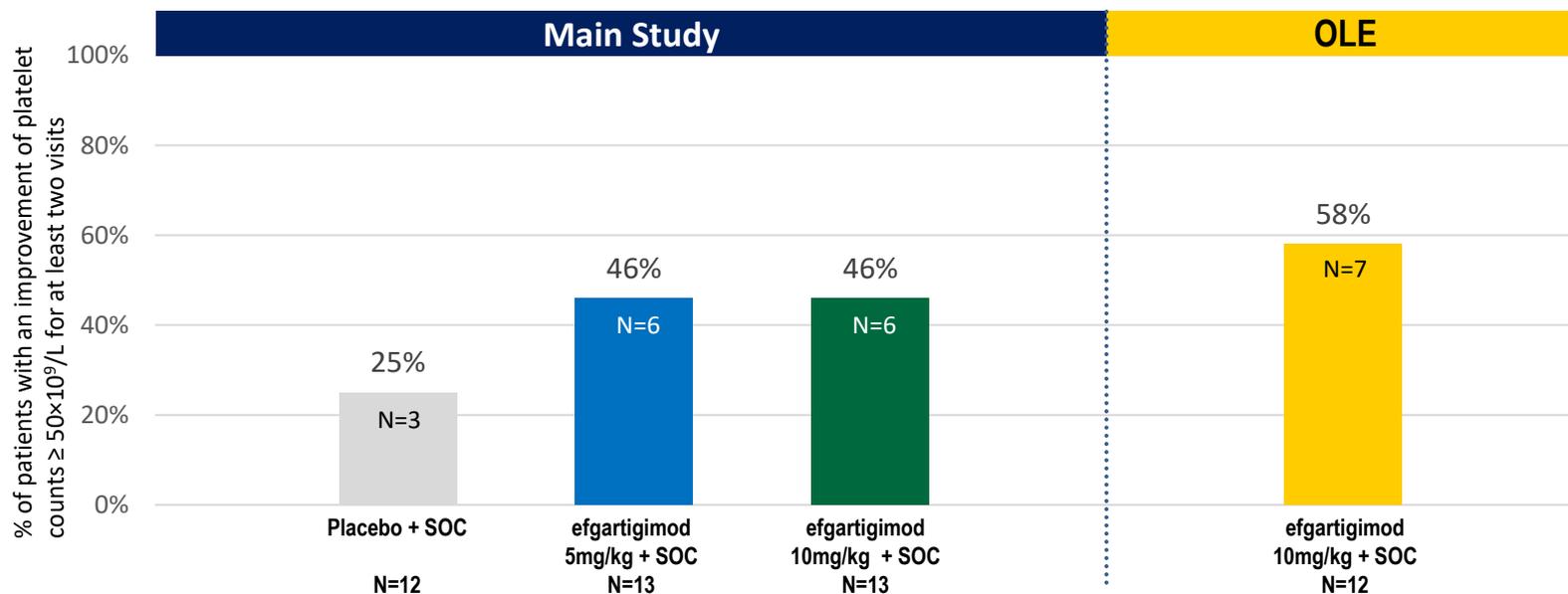
Placebo



Color: patients achieving $\geq 50 \times 10^9/L$, at least two visits. Note: All central lab values for the main study, except for patient marked by (#). All local lab values for the extended follow-up > 78d. Extended follow-up period shown by dotted lines, note frequency of visits dependent on medical need in this period

Strong Improvement of Platelet Counts Across Doses

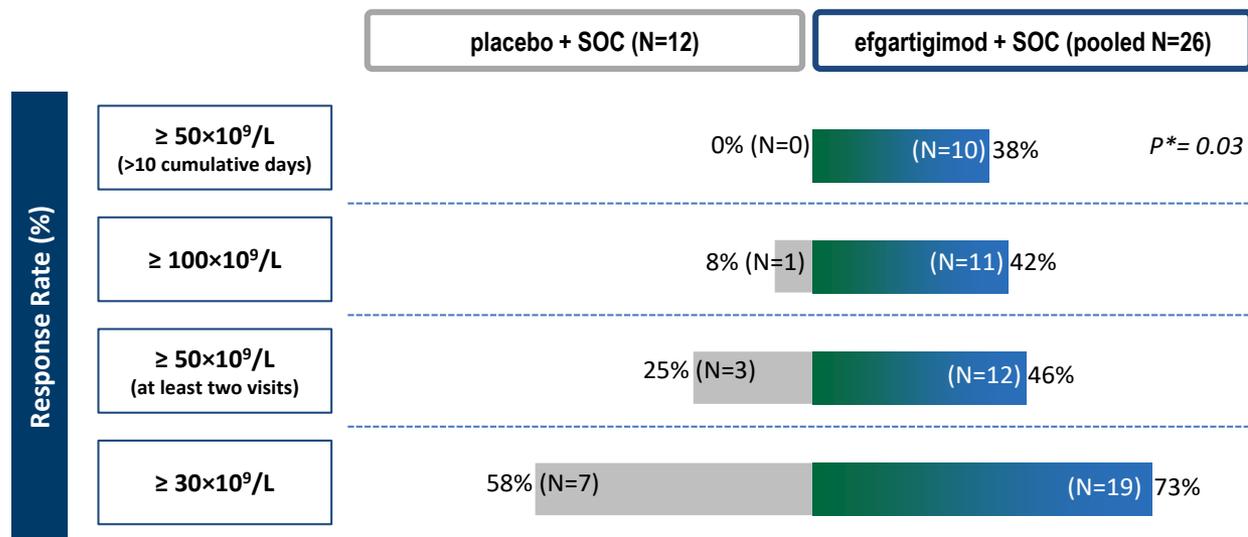
Patients achieving platelet counts of $\geq 50 \times 10^9/L$ at least two times



- 46% of patients for both doses of efgartigimod and 58% of OLE patients realized platelet response $\geq 50 \times 10^9/L$ during at least two visits
- Novel mode of action beyond boosting platelet production or broad immune-suppression

Robust Improvement of Platelet Count

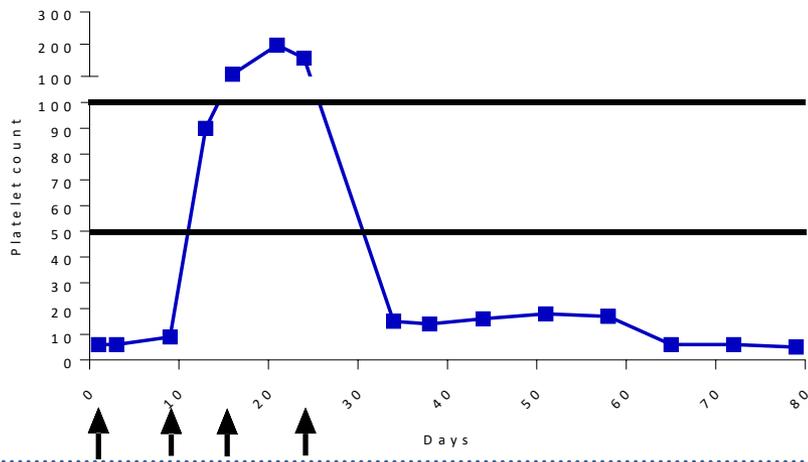
Post-hoc analysis of increasing thresholds of efficacy



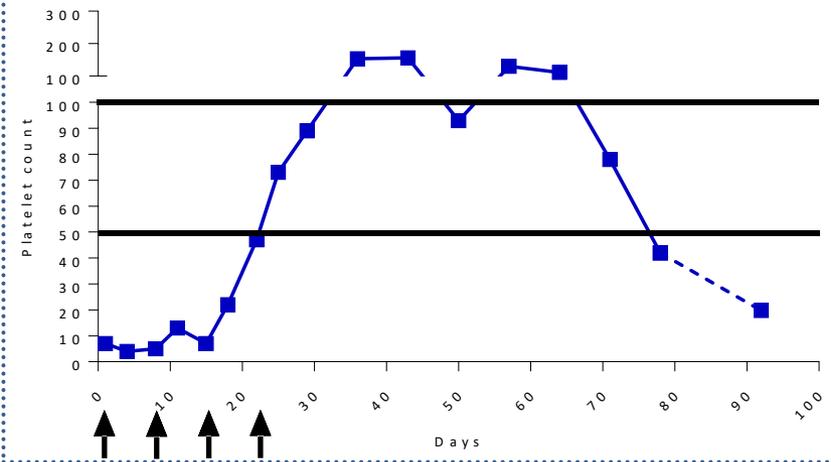
- Efgartigimod generated therapeutic effect at multiple relevant thresholds of efficacy
- Duration of platelets remaining $\geq 50 \times 10^9/L$ ranged from 1 - 20 weeks, with five patients above that platelet threshold for more than a month

Effect Observed Across ITP Classifications and SOC

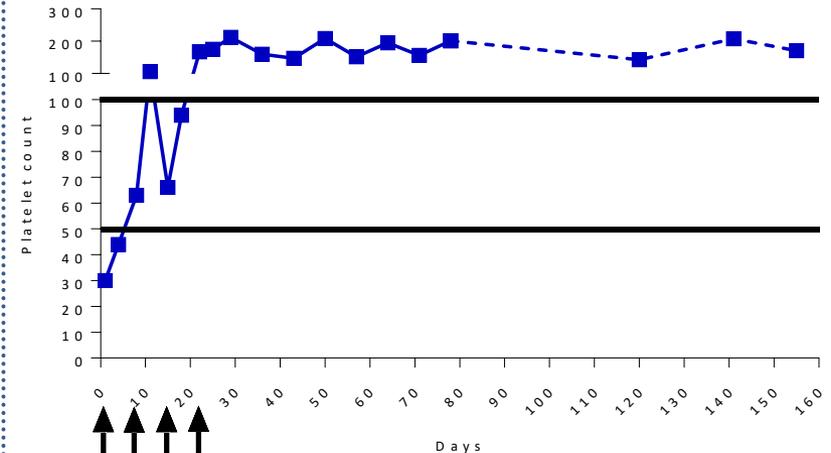
Case 1 (5 mg/kg efgartigimod)



Case 2 (10 mg/kg efgartigimod)



Case 3 (5 mg/kg efgartigimod)



- **Case 1:** Chronic ITP (diagnosed in 1984); Low platelets at base ($<10 \times 10^9/L$); eltrombopag (75 mg); 5 mg/kg efgartigimod
- **Case 2:** Persistent ITP; Low platelets at base ($<10 \times 10^9/L$); watch & wait; 10 mg/kg efgartigimod
- **Case 3:** Newly diagnosed ITP, $\sim 25 \times 10^9/L$ platelets at base; corticosteroids (12 mg) tapering to 4 mg qd in follow-up; 5 mg/kg efgartigimod

Note: central lab values for the main study (until day 78). All local lab values for the extended follow-up $> 78d$. Include extended follow-up for case 2 and 3. Extended follow-up period shown by dotted lines, note frequency of visits dependent on medical need in this period

ITP Phase 2: Hematological Beachhead Established



Favorable and consistent safety & tolerability profile



Clinically meaningful & statistically significant increase of platelet count – across doses and ITP patient types



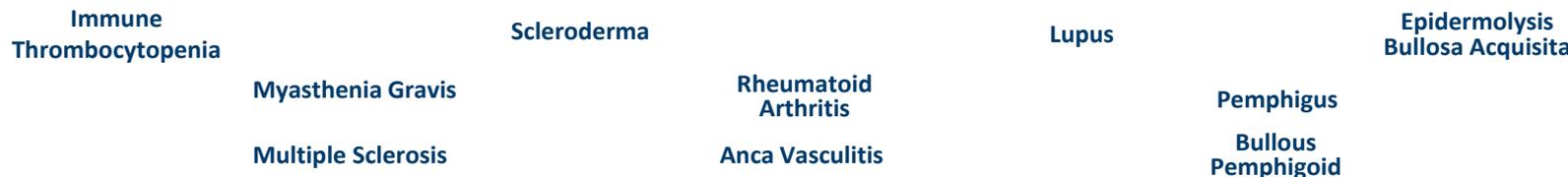
Strong and consistent IgG reduction – validating focus on IgG mediated diseases



Enabling Phase 3 in ITP (IV) and launch of Phase 2 in ITP for SubQ formulation

CIDP Expands the Pipeline-in-a-Product Opportunity

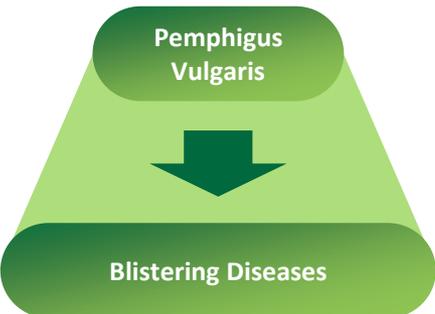
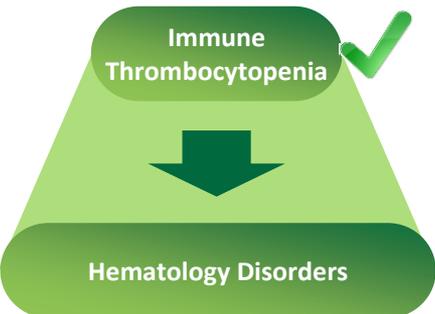
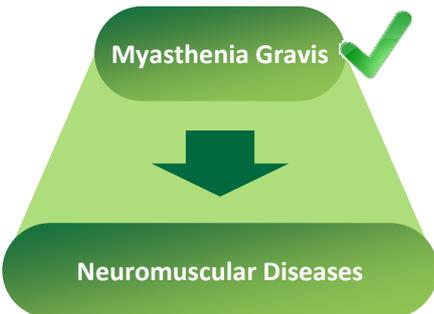
Landscape of IgG-mediated severe autoimmune diseases (sampling)



Solid Biology Rationale
Disease proven to be predominantly mediated by pathogenic IgGs

Feasible for Biotech
Orphan potential, economically viable, efficient clinical & regulatory pathway

Proof-of Concept:



Therapeutic Area Beachheads with Expansion Possibilities into Adjacent Indications

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

What is CIDP?

- Rare, chronic autoimmune disorder of peripheral nerves, nerve roots
 - Caused by destruction of nerve myelin sheath
- Diagnosis based on clinical symptoms and electrodiagnostic findings
- US prevalence: ~16,000 patients; similar number in EU5
- IgG auto-antibodies increasingly identified in patients
- Progressive disease: symptoms include increasing loss of sensation, tingling and pain, loss of reflexes, weakness, difficulty walking, foot drop, and can lead to immobility

Limited treatment options

- IV/SC immunoglobulin *, corticosteroids
 - major IVIg indication **
- Plasma exchange
- Other immunosuppressants

Unmet need in CIDP

- Disease burden significantly underestimated
- Existing treatments are onerous and associated with significant side effects
- New treatments that are more effective and convenient, safer and better tolerated than IVIg or steroids

*Estimated IV/SC Ig US sales in CIDP reached \$1B in 2014

** Robert et al, 2015

NORD; UpToDate; Epocrates; Ann Neurol. 1999;46(6):910.; J Neurol Neurosurg Psychiatry. 1999;66(5):677.; J Neurol Neurosurg Psychiatry. 2007;78(12):1349. Neurology. 2009;73(1):39.; Muscle Nerve. 2009 Apr;39(4):432-8.; Eur J Neurol. 2014;21(1):28-33.

GBS/CIDP Foundation

argenx proprietary market research



“Immune thrombocytopenia is a serious autoimmune condition defined by much more than a low platelet count. Patients describe the constant anxiety they experience from the risk of a bleeding event, the difficult-to-manage side effects from some of the current drugs, and the fatigue from a ‘trial and error’ approach as they cycle on and off available therapies.

September is ITP Awareness Month to serve as a reminder of the ongoing burden patients experience despite a range of available therapies and the need for new modalities as urgent in hopes of alleviating some of this patient suffering.”

Caroline Kruse

Executive Director of the Platelet Disorder Support Association



Thank you!
