Breakfast Symposium Efgartigimod Phase 2 Study in Immune Thrombocytopenia

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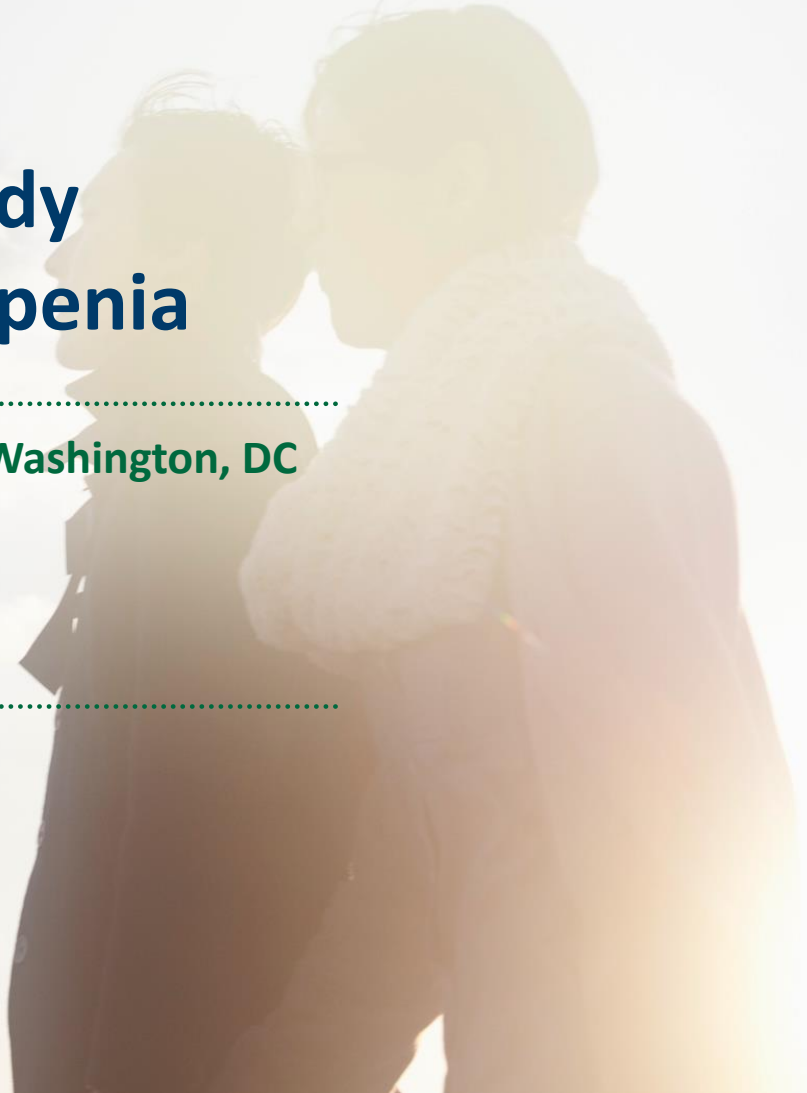
June 19, 2018, New York



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Agenda

08:30 **Welcome & Introduction**
Tim Van Hauwermeiren, argenx

08:35 **Efgartigimod: Phase 2 Study in immune thrombocytopenia**

- **Introduction to immune thrombocytopenia**
  Catherine Broome, MD, Georgetown University, Washington, DC
- **Attractive market opportunity**
  Rebecca Rupert, argenx
- **Strong biologic rationale**
  Peter Ulrichts, PhD, argenx
- **Phase 2 clinical trial in immune thrombocytopenia**
  Tim Van Hauwermeiren, argenx

09:35 **Efgartigimod: Pipeline-in-product opportunity**
Tim Van Hauwermeiren, argenx

09:45 **Q&A**

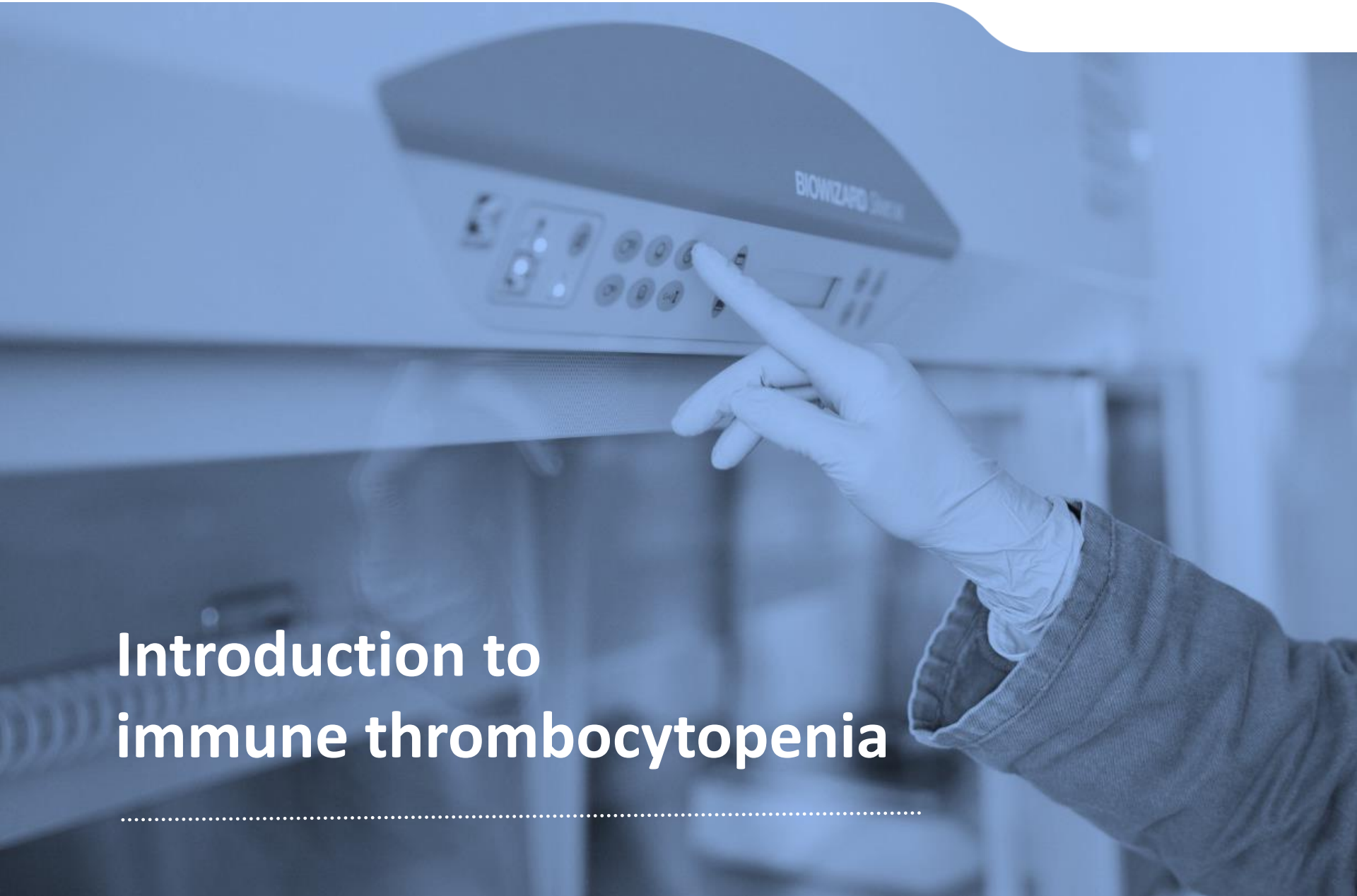
Associate Prof. Catherine Broome

Catherine M. Broome, MD, is board certified in internal medicine, hematology and medical oncology. She is on staff at the MedStar Georgetown University Hospital's Lombardi Comprehensive Cancer Center.

A fascination with the science of hematology and the opportunity to develop a strong relationship with patients and their families led Dr. Broome to specialize in hematology and medical oncology. Dr. Broome focuses on developing a partnership with her patients, providing them the most up-to-date information and treatment options. They work together to develop a treatment plan that fits medically, while also taking in account all aspects of the patient's life.

In addition to patient care, Dr. Broome is also an associate professor in the Lombardi Cancer Center's department of medicine, teaching the next generation of physicians.

Dr. Broome completed medical school at Louisiana State University and completed her internship and residency at Greenville Memorial Hospital. She also completed fellowship programs at Fred Hutchinson Cancer Center and George Washington University.



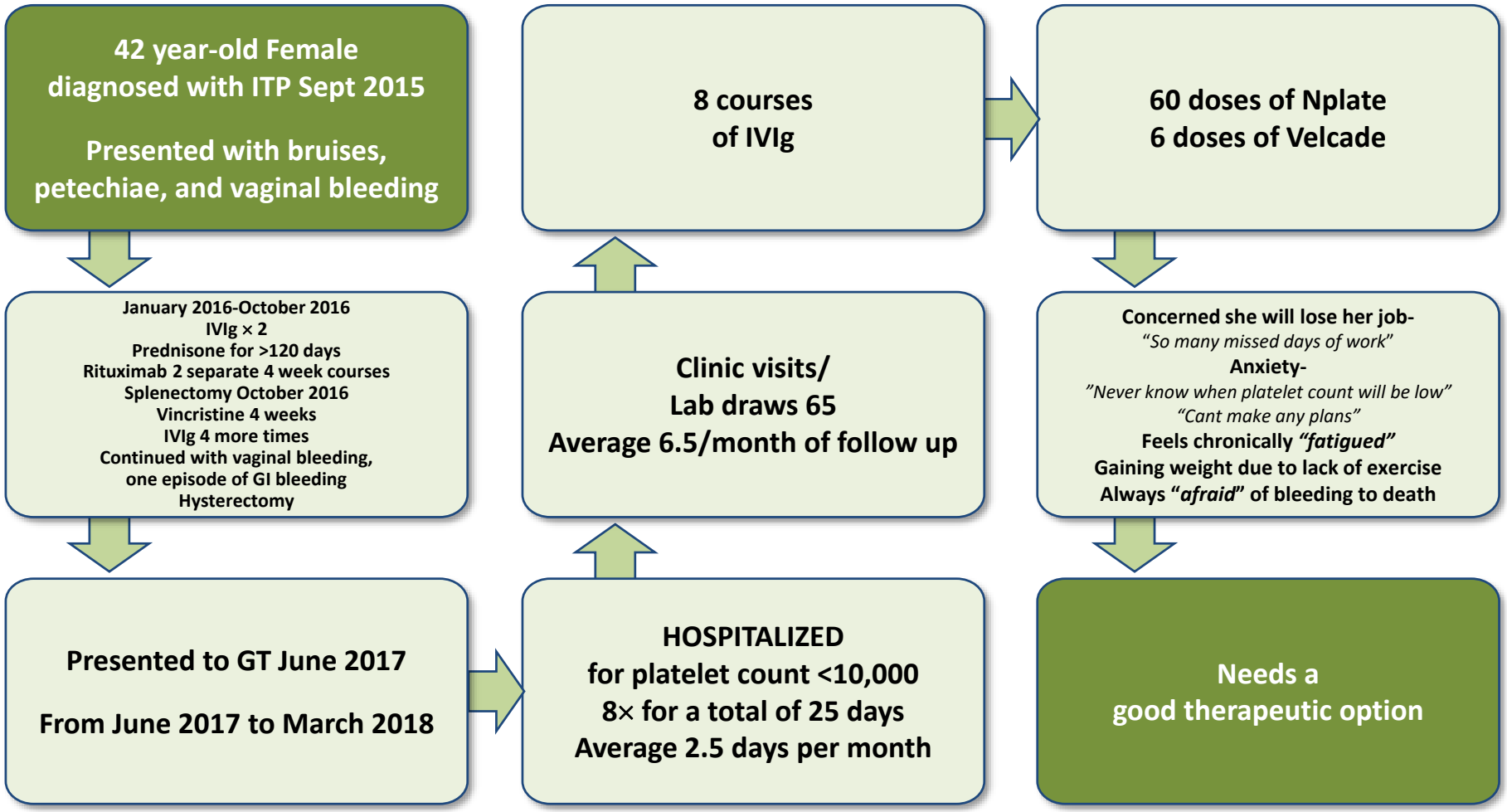
Introduction to immune thrombocytopenia

ITP
Immune Thrombocytopenia

Catherine Broome, MD

Associate Professor of Medicine
Georgetown Lombardi Cancer Center
Washington, DC

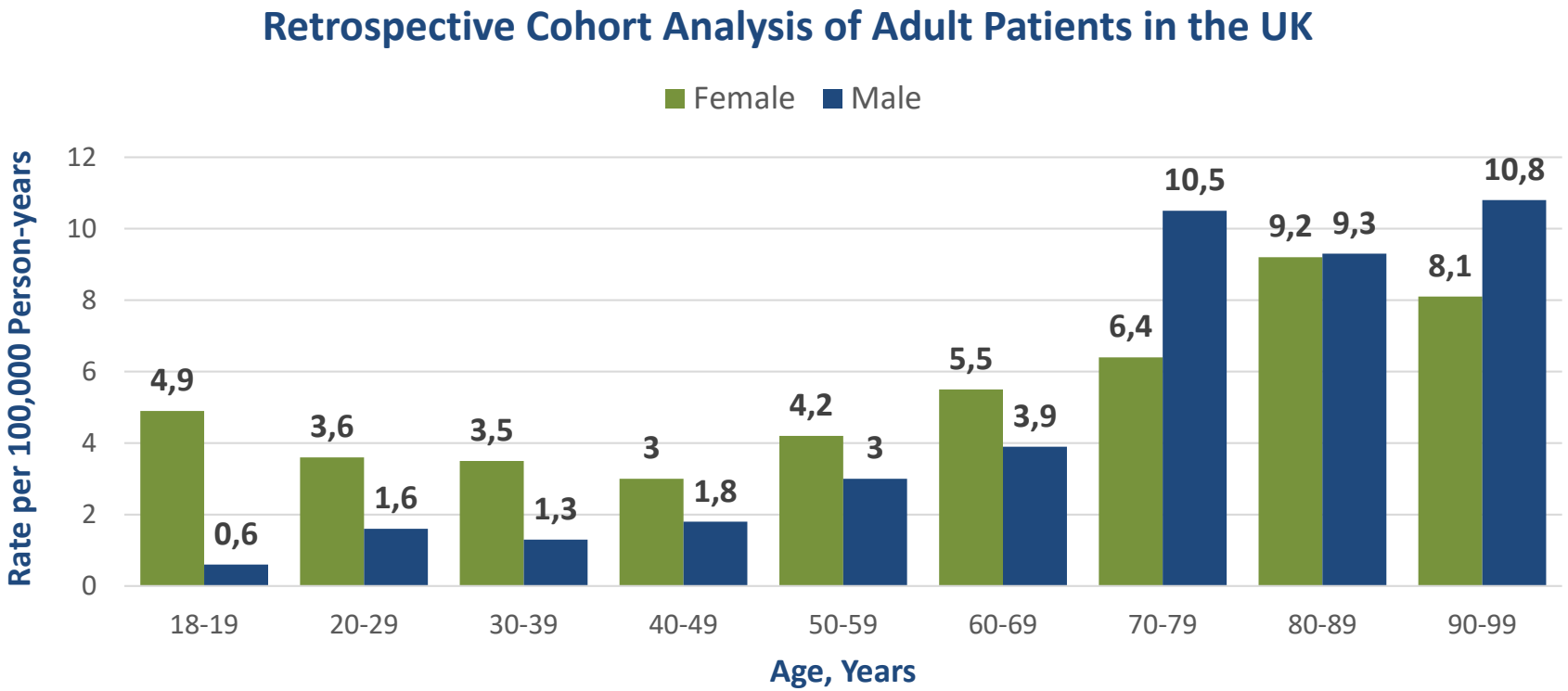
Patient Story



ITP = Immune Thrombocytopenia; IVIg = intravenous immunoglobulin

Incidence of Immune Thrombocytopenia (ITP) in Adults

- Incidence of ITP 3.3/100,000 adults per year
- Prevalence of ITP 9.5/100,000 adults
- More commonly seen in females in the younger adult population but equal frequency between men and women in older adult population



Slide credit: clinicaloptions.com

Abrahamson PE, et al. *Eur J Haematol*. 2009;83:83-89.

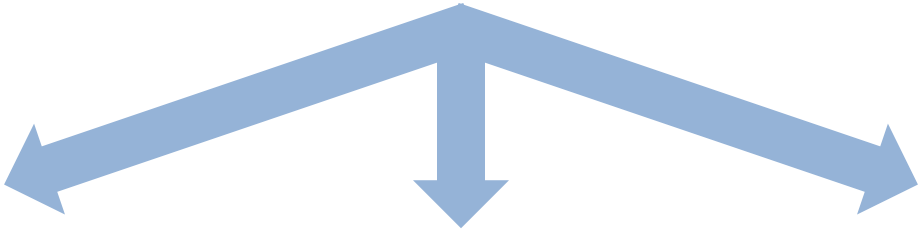
ITP: Not Just a Disorder of Platelet Number

- 1.5 fold higher **mortality** in ITP patients compared to general population
- Bleeding (RR 2.4) and infection (RR 6.2)
- 1.6 xs higher risk of venous thrombosis (VTE) compared to general population
- Physical and psychological manifestations

Morbidity and Mortality in Adults with ITP

Mean 10.5 year follow-up in primary ITP patients

Adults with Primary ITP (N=134)



9% (n=12) had **<30 × 10^9/L**
Refractory disease with severe thrombocytopenia

Mortality risk of 4.2×
(95% CI: 1.7-10.0)

6% (n=8) had **>30 × 10^9/L**
On maintenance therapy

Increased number of hospitalizations but only minimal increase in mortality risk

85% (n=114) had **>30 × 10^9/L**
Off maintenance therapy

Mortality risk mirroring the general population

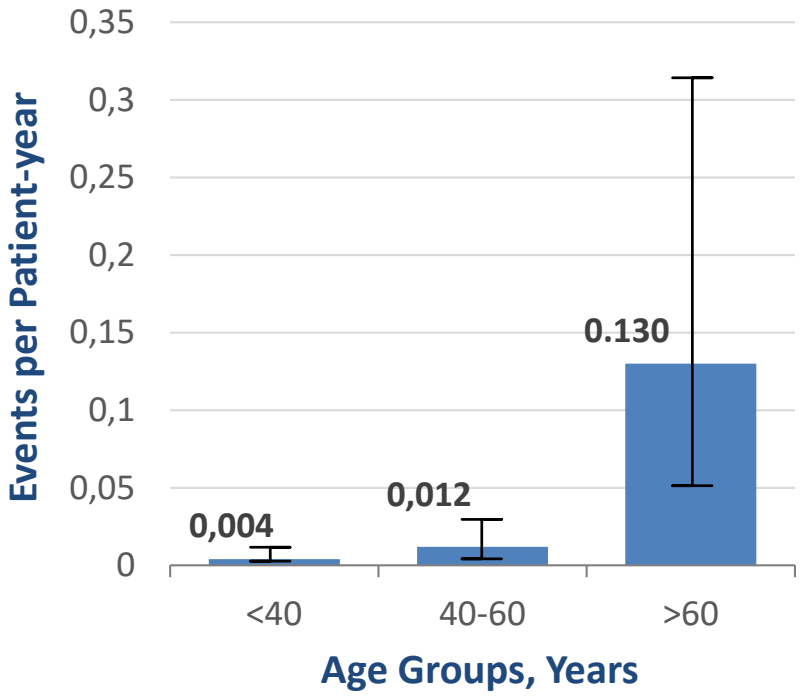
Bleeding and infection contributed equally to mortality

CI = confidence interval
Portielje JE, et al. *Blood*. 2001;97:2549-2554.

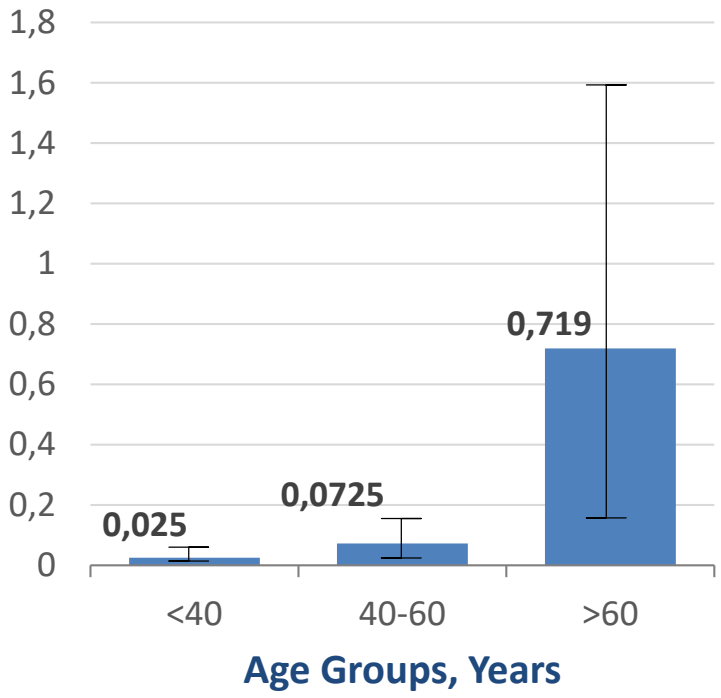
Bleeding in Patients with ITP

Estimated Annual Bleeding Incidence in ITP by Age

Fatal Hemorrhages



Major Nonfatal Hemorrhages



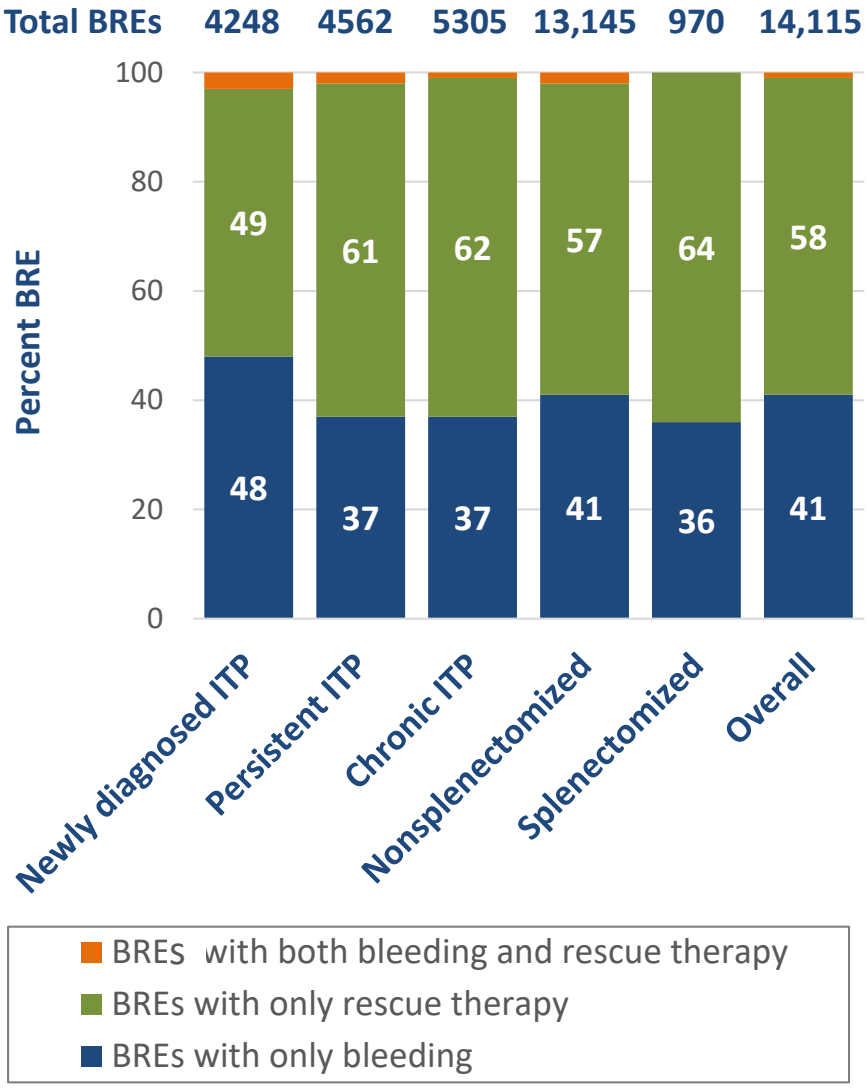
Estimated annual rate of fatal (left) and major nonfatal (right) hemorrhages according to patient age group, based on pooled analysis from idiopathic thrombocytopenic purpura case series. Upper and lower limits represent low and high patient time estimates.

Slide credit: clinicaloptions.com

Cohen YC et al. *Arch Intern Med*. 2000;160:1630-1638.

Bleeding in Patients with ITP

- 6,651 adults diagnosed with primary ITP
- 13,064 patient-years of follow-up
- 3,768 patients (57%) experienced ≥1 BRE (1.08 BREs per patient-year; 95% confidence interval: 1.06-1.10)
- **The majority (58%) of BREs consisted of rescue therapy use only.** Common bleeding types were gastrointestinal hemorrhage, hematuria, ecchymosis, and epistaxis
- **Intracranial hemorrhage was reported in 74 patients (1%)**



BREs = bleeding-related episodes; ITP = immune thrombocytopenia.
Altomare I et al. *Clin Epidemiol*. 2016;8:231-239.

Cost of Bleeding Related Events in ITP

- In 6,551 patients, 14,115 BREs were identified, mean (SD) age was 55 (18) years
- Mean total reimbursement per BRE was $6,022, with significantly higher mean in patient ($45,114) vs outpatient ($2,150) reimbursements (*P*<0.0001)
- Mean reimbursement for BREs associated with bleeding alone was $10,396, and with rescue therapy alone it was $2,787
- Reimbursement for BREs that included both bleeding and rescue therapy was $11,065
- The majority of ITP patients experienced ≥1 BRE, and over half were defined by rescue therapy use alone
- This illustrates the importance of examining both bleeding and **rescue therapy use** to fully assess disease burden

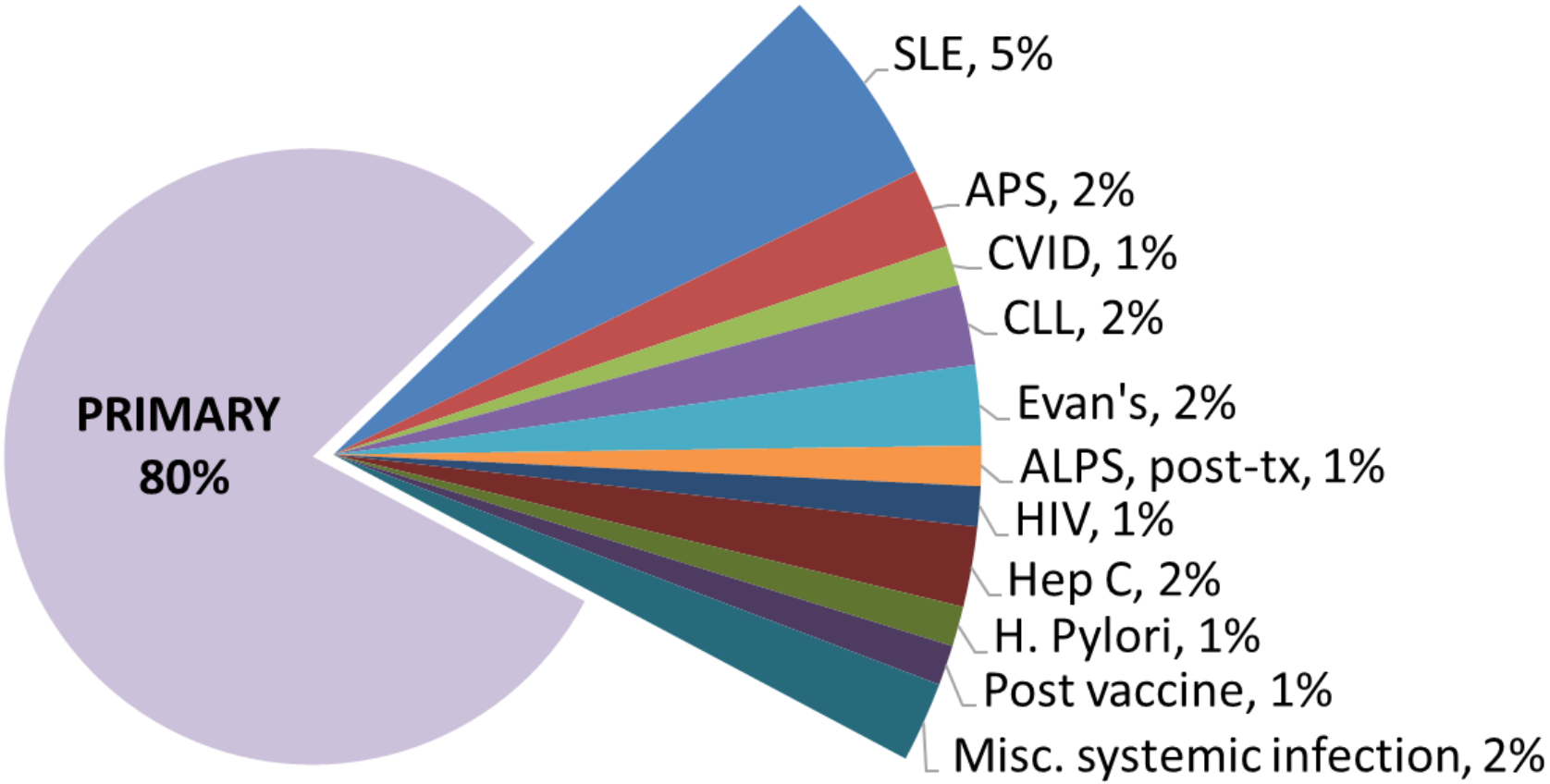
Altomare I et al. *Clin Epidemiol*. 2016;8:231-239.

Immune Thrombocytopenia: Terminology

- **Newly diagnosed** <3 months duration
- **Persistent** 3-12 months duration
- **Chronic** >12 months duration
- **Severe**: Clinically relevant bleeding of sufficient magnitude to mandate treatment or requiring additional interventions or increase in drug dose
- **Refractory:** Presence of severe ITP after splenectomy

Michele P. Lambert and Terry B. Gernsheimer. *Blood*. 2017 ;129 (21):2829-2835.

Immune Thrombocytopenia



Cines DB et al. *Blood*. 2009;113(26):6511–6521.

ITP Response Definitions

- **Response (complete)**: Platelet count >100 X 10^9/L measured on 2 occasions >7 days apart

- **Response**: Platelet count >30 X 10^9/L and a greater than two-fold increase in platelet count from baseline measured on 2 occasions >7 days apart

Michele P. Lambert and Terry B. Gernsheimer. *Blood*. 2017 ;129 (21):2829-2835.

ITP Goals of Therapy ASH Guidelines

Emergency Therapy ITP: IVIg

- IVIg are effective drugs (response rate around 80%) however most responses are short lived (2-4 weeks)
- Recommended as emergency therapy to relatively rapidly increase platelet count to a safe level
- They are usually used concomitantly with steroids to increase their efficacy
- Adverse reactions to IVIG are reported to occur in up to 5 to 15 percent of all IVIG infusions and to affect 20 to 50 percent of individuals receiving IVIg
- Potentially severe reactions include anaphylaxis in some IgA-deficient individuals, thromboembolic events including myocardial and cerebral ischemia, renal impairment, or severe hemolysis
- Usually requires a minimum of 2 day hospital stay

IVIg = intravenous immunoglobulin.

Splenectomy

- Splenectomy is another possibility in the therapy of ITP, with a prolonged response reported as high as in 60-70% and as low as 20-30%
- Not as frequently used today as it has been in past
- The main mechanism of action is the removal of the site of destruction of platelets
- Complications include: risk of infection with encapsulated organisms [especially in the pediatric population], pneumonia, intra- and postoperative hemorrhage, thrombocytosis with or without venous thromboembolism, pancreatitis, gastric fistula
- Mortality ranges from 0 to 9%

Steroid Therapy for ITP

		Dexamethasone Treatment Group	Prednisone Treatment Group			
Study	**Number of Patients**	**Dexamethasone Regimen***	**Prednisone Equivalent†**	**Prednisone Regimen***	**Prednisone Equivalent†**	**6-month Response** (Dexamethasone vs Prednisone)
Wei et al, 2016	192	40 mg/day 3 4 d for 1-2 cycles	14.2 mg/kg per cycle	1 mg/kg per day for 28 d	28 mg/kg	**40.0% vs 41.2%** (*P*=0.884); platelet count >30 X 10^9/L with an absence of bleeding and no additional treatment
Bae et al, 2010	151	40 mg/day 3 4 d for 1-2 cycles	14.2 mg/kg per cycle	1 mg/kg per day for 28 d	28 mg/kg	**33.3% vs 45.0%** (*P*= 0.33); platelet count >30 X 10^9/L
Din et al, 2015	94	0 mg/day 3 4 d for 3 cycles with maintenance 0.035 mg/kg/day dexamethasone between cycles (n 5 30) or without maintenance (n 5 31)	42.8 mg/kg	1 mg/kg per day for 28 d	28 mg/kg	**74.1% with maintenance (*P* <.05) vs 60% without maintenance vs 58.8%;** platelet count >30 x 10^9/L and at least double baseline without bleeding
Mashhadi et al, 2012	60	40 mg/day 3 4 d for 1 cycle‡	14.2 mg/kg	1 mg/kg per day for 28 d	28 mg/kg	**90% vs 53.3%** (*P*< 0.0001); platelet count >30 X 10^9/L

Other Therapeutic Interventions for ITP

- Rituximab
- Splenectomy
- Thrombopoietin receptor agonists
  - Romiplostim
  - Eltrobopag
- Mycophenylate mofetil
- Vinca alkaloids
- Azathioprine
- Cyclosporine A
- Cyclophosphamide
- Danazol
- Dapsone
- Tavalisse (fostamatinib)

Provan et al. *Blood*. 2010;115:168-186.

Treatment of Adult ITP

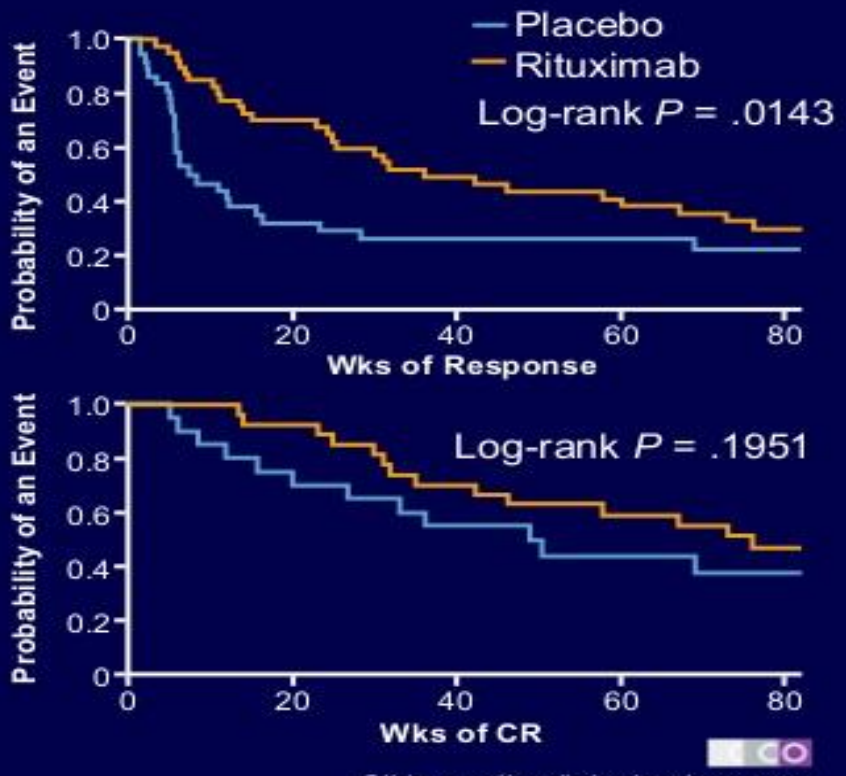
Treatment	Dose	Time to Initial Response, Day	Time to Peak Response, Day
Rituximab	375 mg/m2/dose iv (4 weekly dose)	7-56	14-180
Splenectomy		1-56	7-56
Vincristine	Up to 2 mg/dose iv (4-6 weekly doses)	7-14	7-42
Vinblastine	0.1 mg/dose iv (6 weekly doses)	7-14	7-42
Danazol	400-800 mg po OD	14-90	28-180
Azathioprine	2 mg/kg po OD	30-90	30-180
Romiplostim	3-10 µg/kg weekly SC	5-14	14-60
Eltrombopag	25-75 mg po OD	7-28	14-90

Rodeghiero F et al. *Blood*. 2009;113:386-393; Provan et al. *Blood*. 2010;115:168-186.

Is Rituximab better than Placebo for ITP?

RITP: Rituximab vs Placebo in ITP Relapse

- Rituximab used as a second-line treatment in randomized, double-blind, multicenter, placebo-controlled trial



Ghanima W, et al. Lancet. 2015;385:1653-1661.

Slide credit: clinicaloptions.com

Thrombopoietin Receptor Agonist Therapy for ITP

- Thrombopoietin receptor agonists (TPO-RAs) have a initial response rate as high as 70–80% but longer term responses at only 30 – 40%

- TPO's main mechanism of action is to increase platelet production by megakaryocytes

- Romiplostim and eltrombopag are currently available

Incidence of Adverse Events in Patients Receiving Long-term Romiplostim Treatment for Chronic ITP

	Cohort 1 (n=33)	Cohort 2 (n=88)	Cohort 3 (n=31)	Cohort 4 (n=139)	Total (n=291)
Any AE, n (%)	32 (97)	88 (100)	30 (97)	134 (96)	284 (98)
Serious AE, n (%)	18 (55)	40 (46)	14 (45)	45 (32)	117 (40)
Treatment-related AE, n (%)	18 (55)	37 (42)	10 (32)	38 (27)	103 (35)
Treatment-related serious AE, n (%)	8 (24)	3 (3)	5 (16)	8 (6)	24 (8)
Deaths, n (%)	0 (0)	6 (7)	3 (10)	7 (5)	16 (5)
Thrombotic events, n (%)	5 (15)	5 (6)	8 (26)	7 (5)	19 (7)
Bleeding events, n (%)	30 (91)	63 (72)	23 (74)	50 (36)	166 (57)

Kuter DJ, Bussel JB, Newland A, et al. *Br J Haematol*. 2013;161(3):411-423.

Long Term TPO in ITP

- Data from 14 studies conducted between July 2002 and June 2011 were included
- A total of 921 patients had received romiplostim for up to 5.4 years for a total exposure of 1,520 patient-years with a mean treatment duration of 76 weeks
- The mean weekly dose of romiplostim was 4.2 µg/kg
- The most frequent duration-adjusted adverse events with romiplostim in this composite analyses included headache, contusion, epistaxis, and nasopharyngitis
- **The rate of thrombotic events was 5.5/100 patient-years.** The most common types of thrombotic events were deep venous thrombosis (1.1/100 patient-years), pulmonary embolism (0.9/100 patient-years), and myocardial infarction (0.6/100 patient-years). Thrombotic events occurred across a wide range of platelet counts and did not appear to correlate with time above platelet thresholds
- Increased bone marrow reticulin was reported in 17 patients and increased bone marrow collagen in one patient receiving romiplostim

Prakash Vishnu and David M Aboulafia. *J Blood Med*. 2016;7:99-106.

Fostamatinib: An Oral Syk Inhibitor

- In two parallel, phase 3, multicenter, randomized, double-blind, placebo-controlled trials (FIT1 and FIT2), patients with persistent/chronic ITP were randomized 2:1 to fostamatinib (n=101) or placebo (n=49)
- Stable responses occurred in 18% of patients on fostamatinib vs 2% on placebo (*P*=.0003)
- Median time to response was 15 days
- The most common adverse events were diarrhea, hypertension, nausea, dizziness, and ALT increase

Bussel J, Arnold DM, et al. *Am J Hematol*. 2018 Apr 26. doi: 10.1002/ajh.25125. [Epub ahead of print].
Fostamatinib for the treatment of adult persistent and chronic immune thrombocytopenia: Results of two phase 3, randomized, placebo-controlled trials.

Non Bleeding Symptoms/QoL

Signs and Symptoms

Emotional Health / Fatigue / Relationships

- Fear, Stress, and Anxiety
- Fear of low platelet counts
- Fear of accidents
- Fear of intracranial bleeding
- Financial stress
- Stress contributes to low platelet levels
- Fear of dying
- Inability to get out of bed
- Spouse
- Limits daily activities
- Embarrassment
- Depression, Isolation, Loss of control
- Suicidal
- Anxiety about medical profession's lack of knowledge

Other

- Migraines
- Visual impairment
- Blood blisters
- Bruising/bruises that never go away
- Bruises all over legs
- Petechia
- Joint aches

Non Bleeding Symptoms/QoL

Functional

Health, Social, Daily Activities / Sports, Exercise and Physical Activities

- Housework, including cooking
- Fatigue limits daily activities
- Extreme care in doing simple tasks
- Unable to do sports, boxing, martial arts, skydiving, climbing, dancing
- Unable to go to the gym

Leisure Activities / Changes in Lifestyle

- Social stigma
- Not comfortable
- Unable or too tired to go out with friends
- Restlessness
- Inability to plan for the future
- Hide the severity of disease from family
- Tired, but unable to sleep
- Travel is limited or more difficult
- Reduction in risk-taking activities
- Feelings of isolation due to physical and emotional effects of ITP
- People suspect spousal or parental abuse due to bruising

Reproductive Health / Women's Reproductive Issues

- Hysterectomy because of bleeding
- Heavy menstrual bleeding
- Inability to have children
- Change in Attitudes/Sex
- Bleeding/bruising
- Reduced libido

Career Advancement / Work Life and Absences

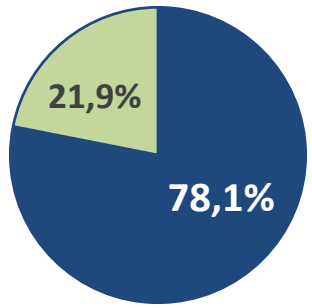
- Lost promotions
- Work is not as satisfying
- Fatigue hinders work
- Low productivity
- Working part-time due to absences
- Unable to pursue desired career
- Permanent disability
- Frequent absences due to illness, and due to medical visits
- Work in a low risk environment
- Work to support family
- Work is a lower priority
- Unemployed

The Comparison of Perceived Stress in Idiopathic Thrombocytopenic Purpura Patients*

Mental stress and daily crises comprise a part of physical and mental threats

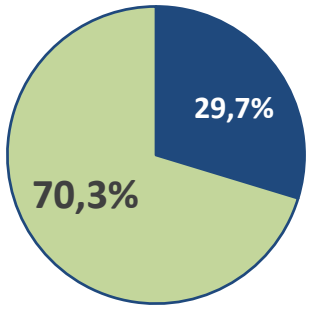
Perceived stress is a physical and mental threat, as well. Perceived stress is a psychological process during which the individual considers his/ her physical and psychological welfare as being threatened

Severe Perceived Stress†



Case group, n=64 Control group, n=64

Mild Perceived Stress†



Case group, n=64 Control group, n=64

Mann-Whitney test showed significant difference between two groups in level of stress (P<0.001).

*64 ITP patients referred to Seyed Al-Shohada in Isfahan, Iran and 64 healthy individuals from the patients' neighborhood, as the control group, were selected randomly and compared in 2013. 64.1%, 59.4% and 53.1% of participants in case group were older than 35 years old, female, and had elementary education.

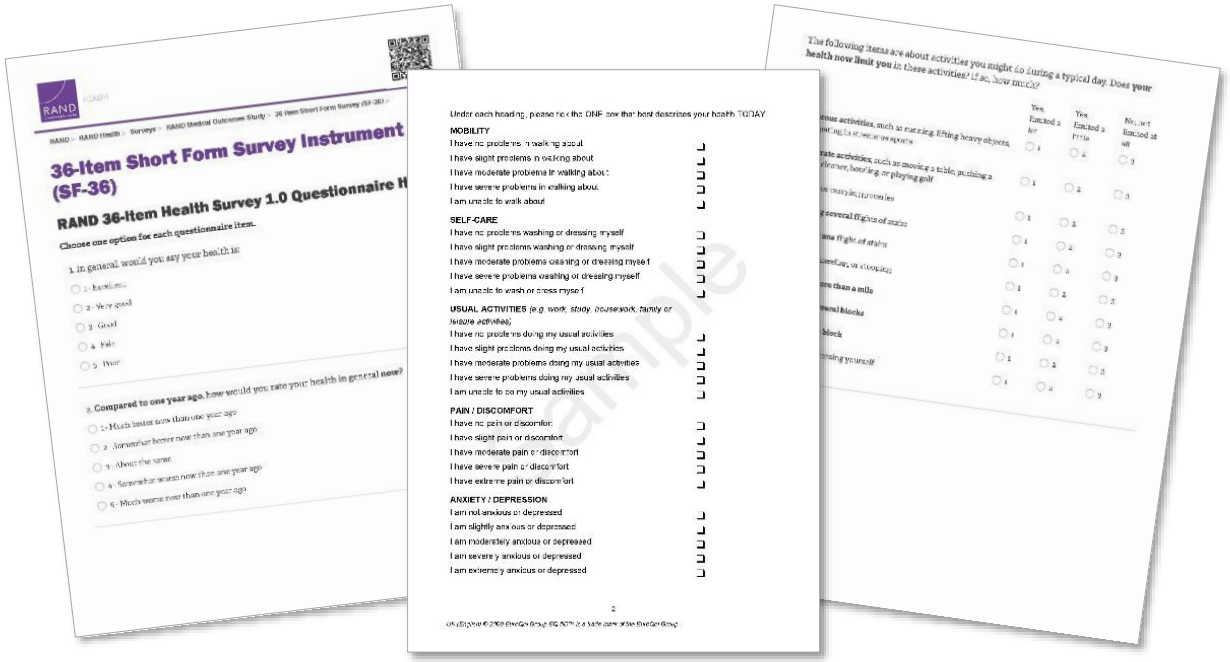
†The Kohen Perceived Stress Standard Questionnaire was used to collect the data. The data were analyzed by SPSS and Student's independent t-test, chi-square, and Mann-Whitney test.

Hemati Z, Kiani D. *Int J Hematol Oncol Stem Cell Res*. 2015 Apr 1;9(2):67-71.

Health-related Quality of Life of ITP Patients: Results from a Web-based Survey

Age and gender matched: 1002 ITP patients and 1031 controls

- ITP patients scored worse on
  - Seven of eight SF-36 domains
  - Physical and Mental Summary scores (all $P<0.05$)
  - EQ-5D visual analog scale (65.5 vs. 82.3; $P=0.002$)
- ITP patients who had undergone splenectomy had similar SF-36 and EQ-5D scores to non-splenectomy patients
  - Scored significantly worse on 5 of 10 ITP-PAQ scales—Bother, Psychological, Fear, Social Activity, and Work (all $P<0.05$)



Snyder CF et al. *Curr Med Res Opin*. 2008 Oct;24(10):2767-2776. doi: 10.1185/03007990802377461. Epub 2008 Aug 19.

Take Away

- ITP should be considered a syndrome, not a SINGLE DISEASE
- ITP is NOT just a disorder of platelet number but is a systemic disease
- The many possible pathways leading to **thrombocytopenia** are different from one patient to another
- These differences account for the different responses to treatments
- They also account for the varied SYMPTOM COMPLEX in ITP patients

Take Away

- Although a better understanding of the immune response at the individual patient level will lead to better use of treatments
- There are MANY patients whose ITP syndrome is “under treated”, poorly managed, or not responsive to currently available therapy
- **There is a REAL need for better ITP therapy**

Attractive market opportunity

Living with ITP Significantly Impacts Patient's Lives

Nancy, diagnosed 5 years ago at age 40

4 MDs consistently seen
5 visits to hematologist on average a year

16 rounds of IVIg

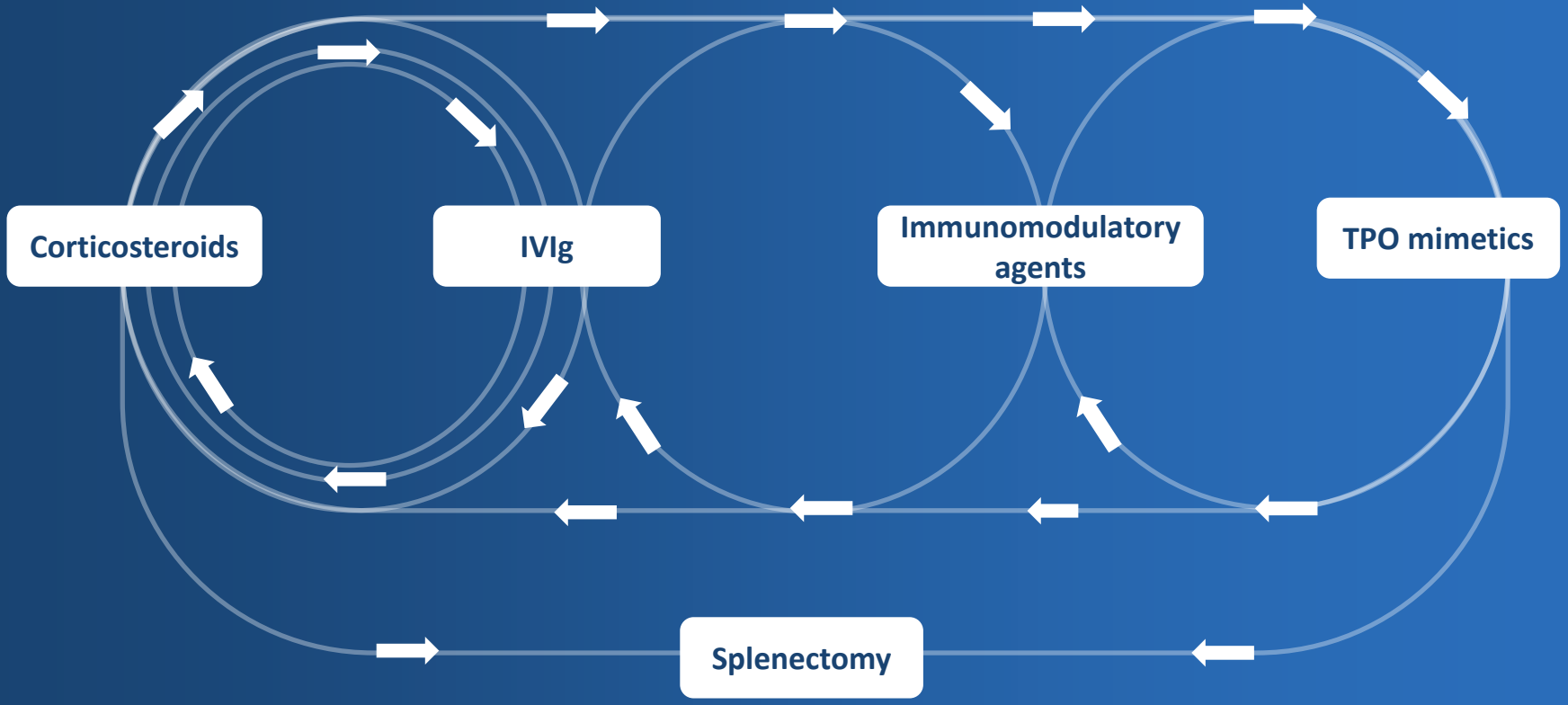
Over **1,000** days on Prednisone

3 hospital admissions with 40 days in patient

120 days, on average, she says she doesn't feel well each year

Patients Cycle through Multiple Treatment Options

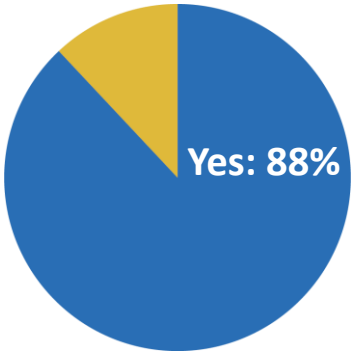
Medication changes over time with frequent side effects



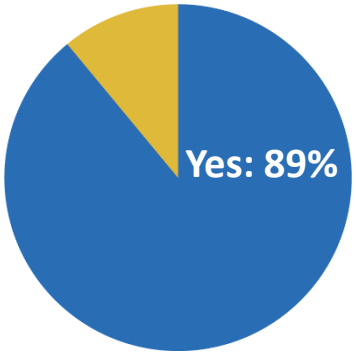
Current Medical Research and Opinion, 25:12, 2961-2969

Significant Impact on Emotional and Functional Health

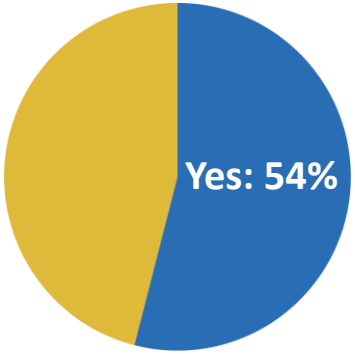
Fatigue



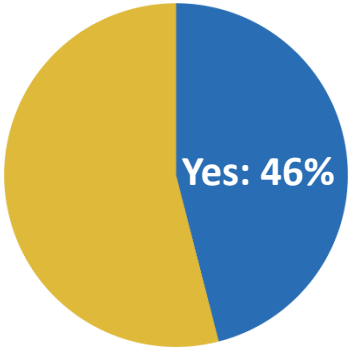
Depression



Anxiety



Emotional impact



ITP Natural History Study Registry; November 2017

Platelet Disorder Support Association Advocacy with FDA

Prioritization of Unmet Needs

PDSA contacted the FDA to meet with OHOP and PASE

Platelet Disorder Support Association 20 Years
Empowering ITP Patients

Executive Director of PDSA and ITP patient comment:

"… therapy options exist, but are often accompanied by **side effects**, tolerability and **toxicity issues**…"

"… **prioritize the unmet needs** of the ITP community….treatments that **last** and provide **better quality of life**…"

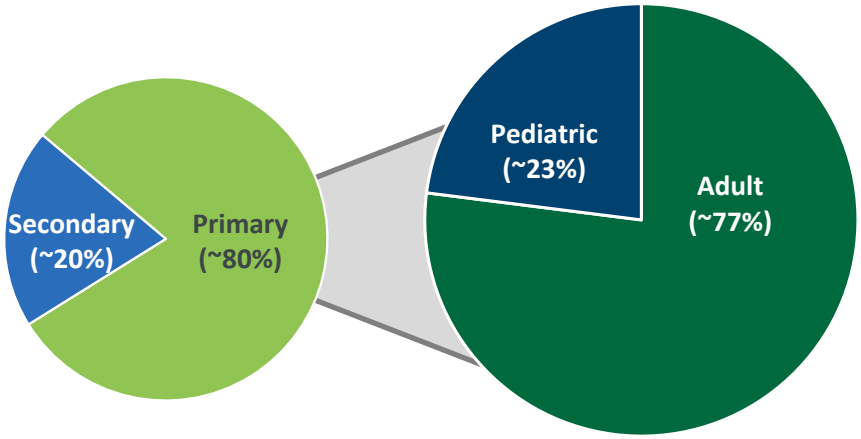
PDSA advocates for more treatment options for patients with ITP:

"… ITP not only **impacts quality of life**, but also can be **life threatening**…"

"The availability of a **new treatment option** provides the ITP community with **more choices**." *~Extract Rigel Press Release*

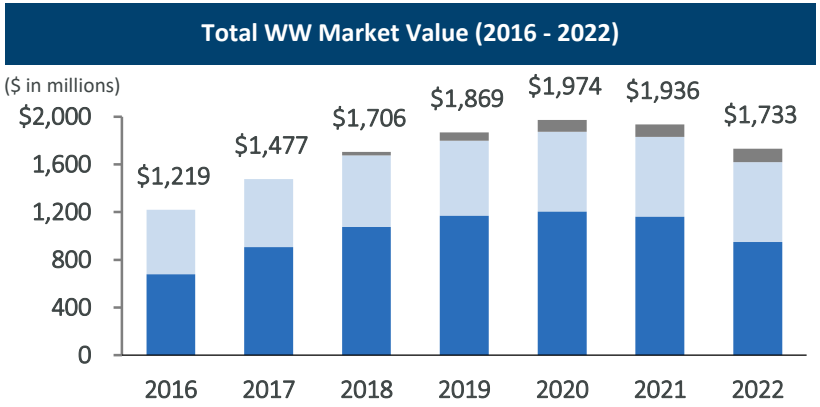
Continued Growth Projected in ITP Market

ITP Prevalence



Estimated WW Sales of Key Competitive Products

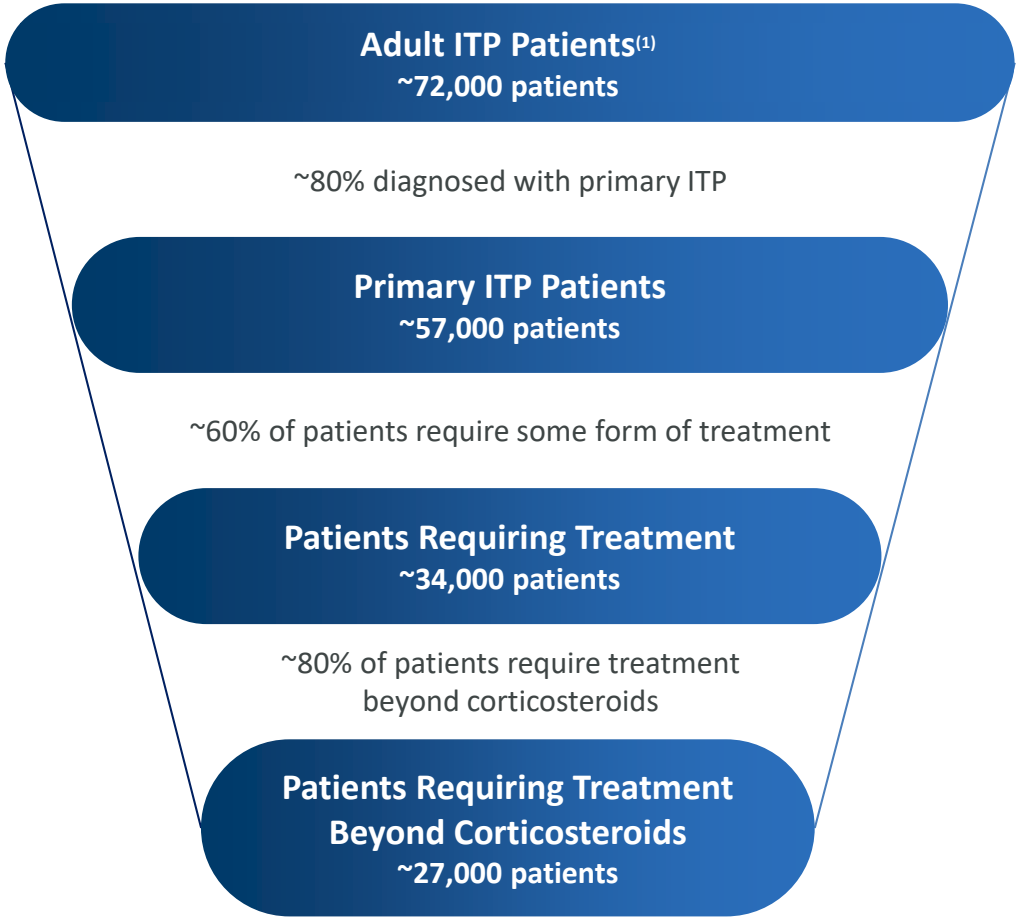
Product Candidate	Company	Stage	2022 Revenue
Promacta	NOVARTIS	Marketed	$948MM
Nplate	AMGEN	Marketed	$671MM
Tavalisse	RIGEL	Approved	$114MM



1) Michael Lambert, Terry Gernsheimer. Clinical updates in adult immune thrombocytopenia. 2017.
2) Donald Arnold, et al. Misiagnosis of primary ITP and frequency of bleeding. 2017.
3) Shruti Chaturvedi, Donald Arnold, Keith McCrae. Splenectomy for immune thrombocytopenia: down but not out. 2018.
4) Shosaku Nomura. Advances in Diagnosis and Treatments for Immune Thrombocytopenia. 2016.
5) Drew Provan, Adrian Newland. Current Managment of Primary Immune Thrombocytopenia. 2015.
6) Wall Street research.

Significant Addressable Market in the U.S.

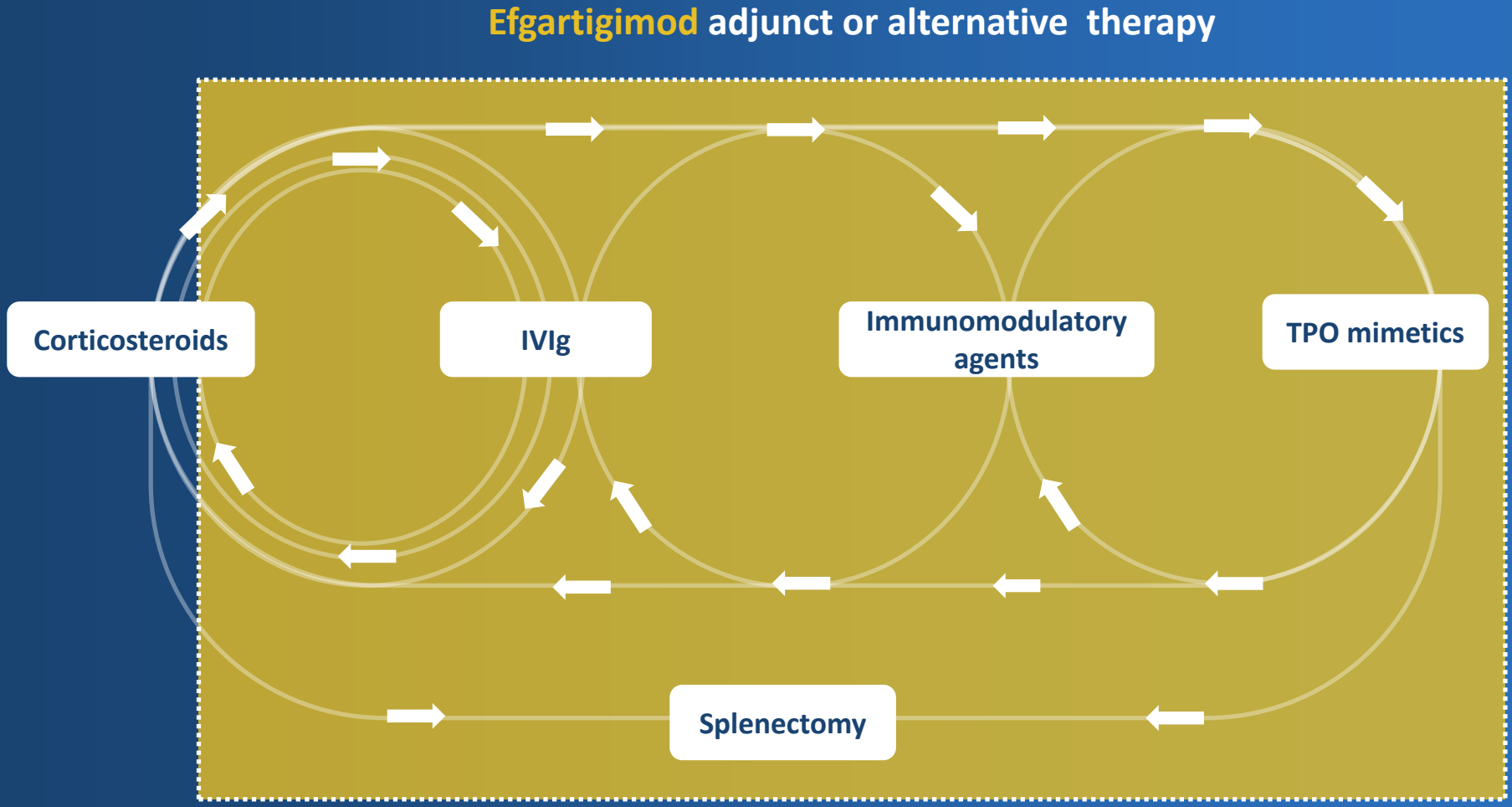
Adult ITP U.S. Market Landscape



Efgartigimod addressable market represents ~27,000 adult primary ITP patients in the U.S.

(1) Saleh et al. 2015, Curr Med Res Opin.; Terell et al. 2012, Am J Hematol.; Grace et al. 2012, Pediatr Blood Cancer

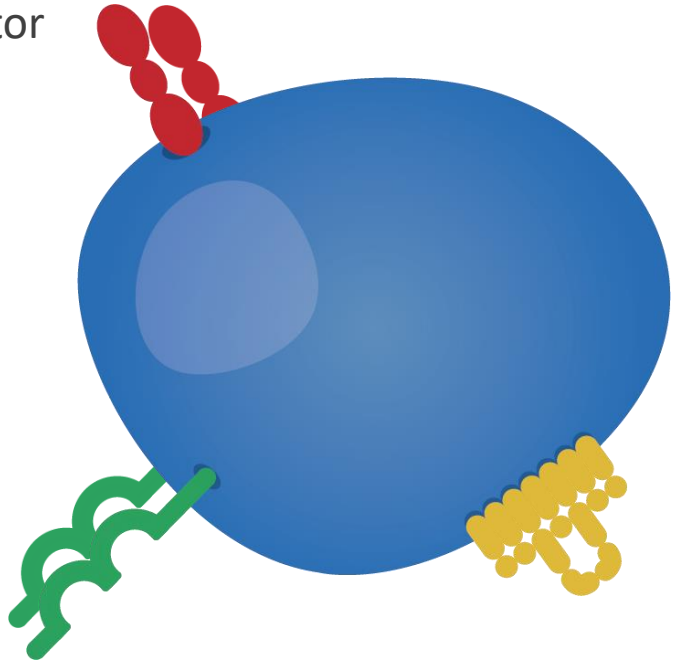
Projected Use of Efgartigimod in the Treatment Cycle



Current Medical Research and Opinion, 2009, 25:12, 2961-2969

Autoantibodies: key mediators of ITP pathophysiology

Platelet Membrane Glycoproteins Are the Autoantigens in ITP

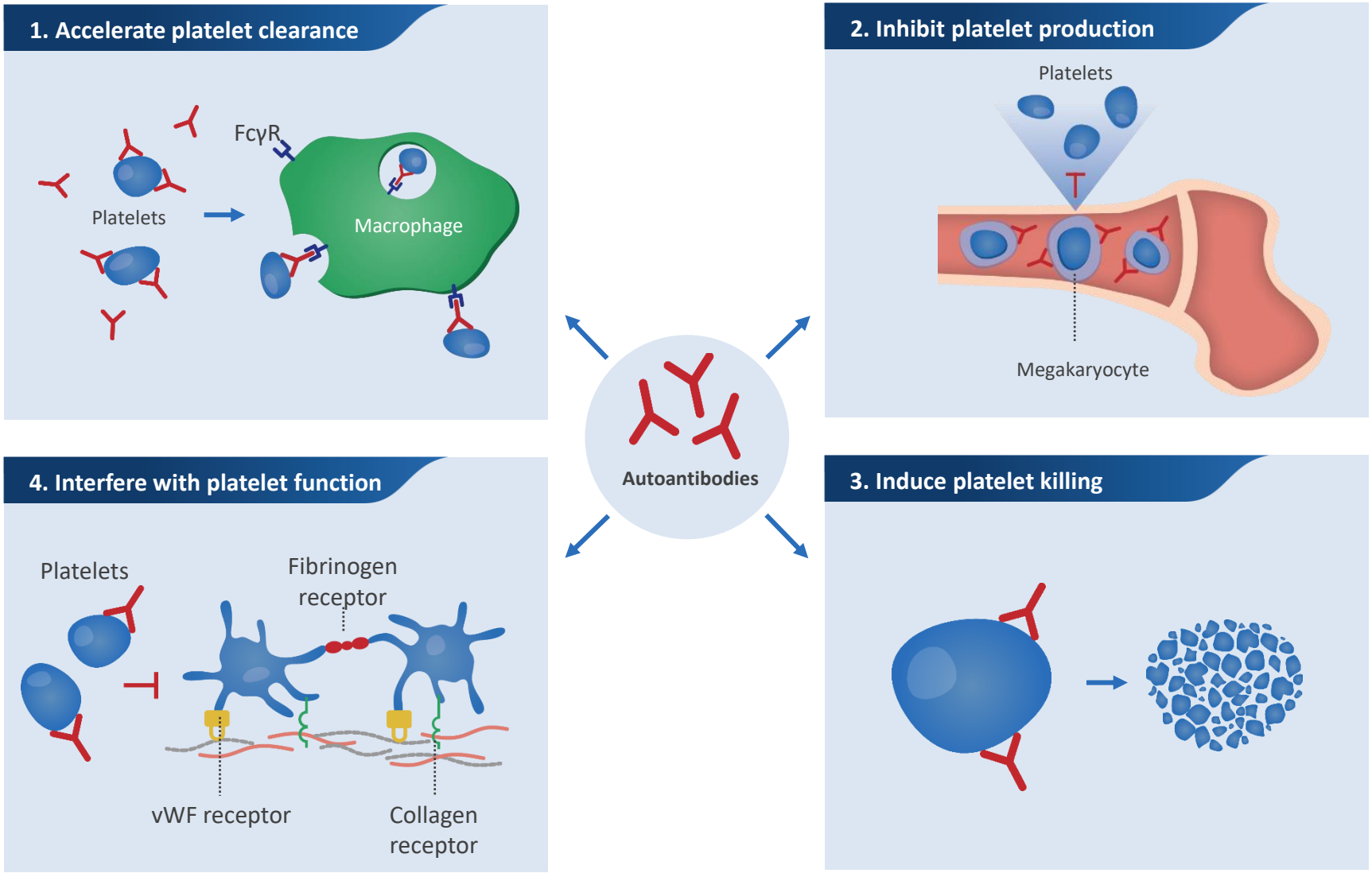


The membrane glycoproteins (GP) of human platelets mediate:

- adhesion to the sub-endothelial matrix: GPIb/IX/V complex (vWF receptor) and GPIa/IIa (collagen receptor)
- platelet-platelet aggregation: GPIIb/IIIa (fibrinogen receptor)

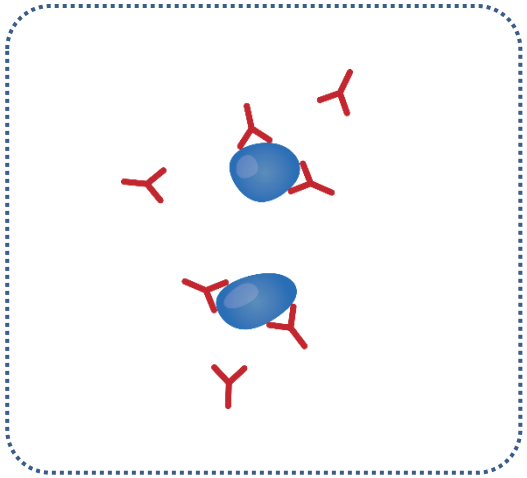
Adapted from Zapata, 2014, PLOS

Autoantibodies: Central Mediators of ITP Pathophysiology

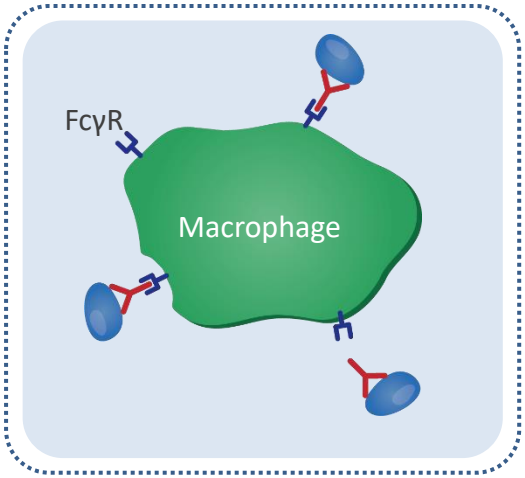


MoA 1: Autoantibodies Accelerate Platelet Clearance

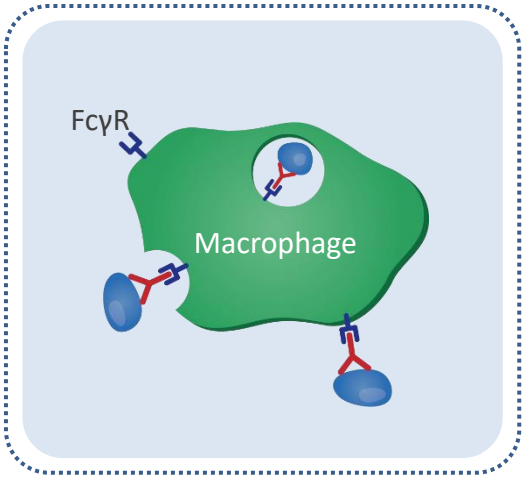
Autoantibodies opsonize platelets...



...resulting in FcγR interaction on macrophages...

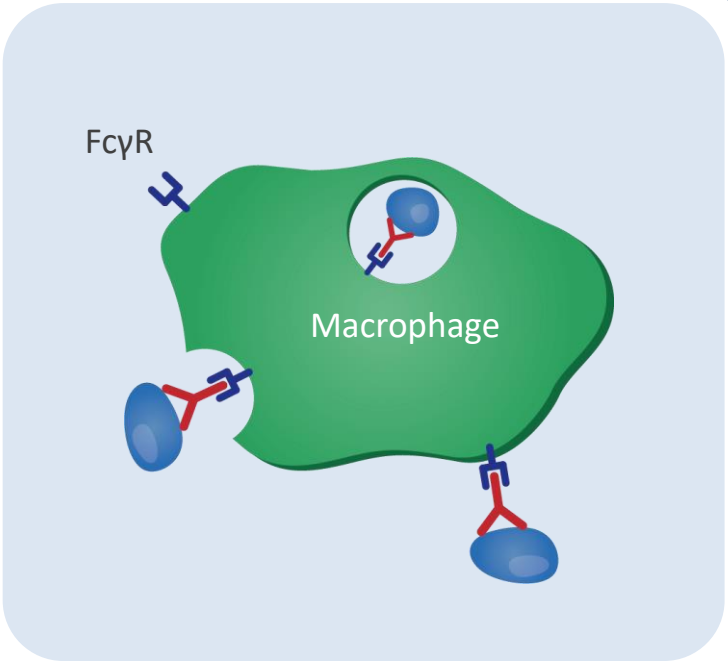


...leading to phagocytosis in spleen and liver.

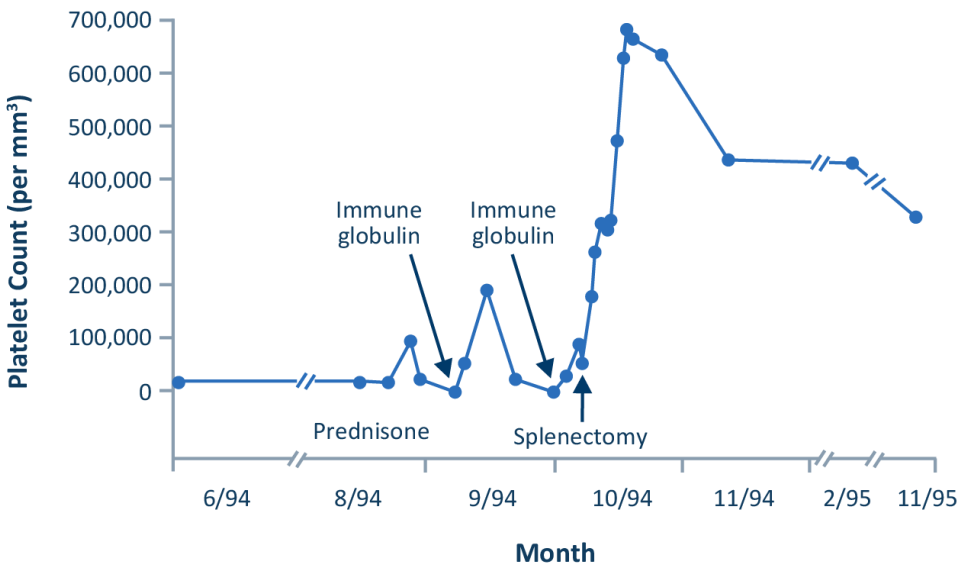


MoA 1: Autoantibodies Accelerate Platelet Clearance in ITP

FcγR-mediated platelet clearance in spleen and liver...



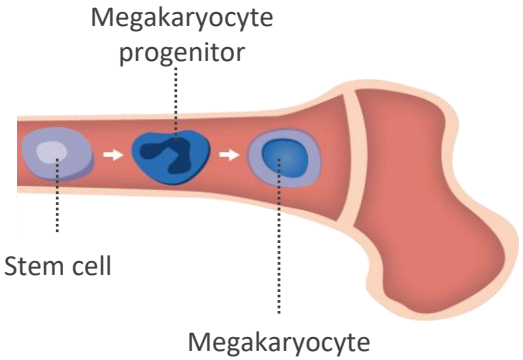
...explaining response to splenectomy and IVIg treatment



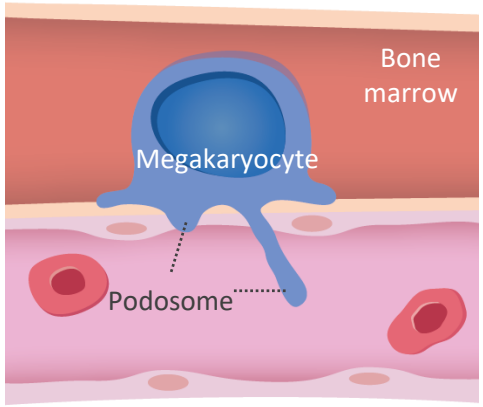
Law et al., 1997, NEJM

MoA 2: Autoantibodies Inhibit Platelet Production

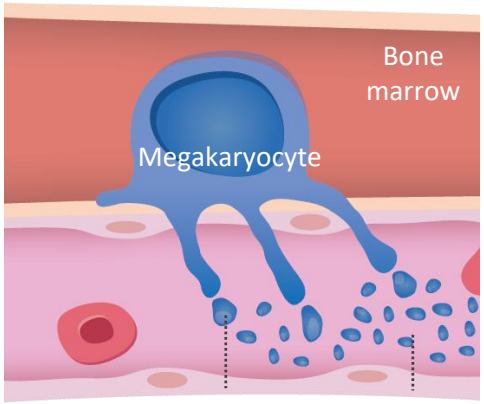
Megakaryocytes mature in the bone marrow



Megakaryocytes form podosomes and proplatelets in vascular lumen...



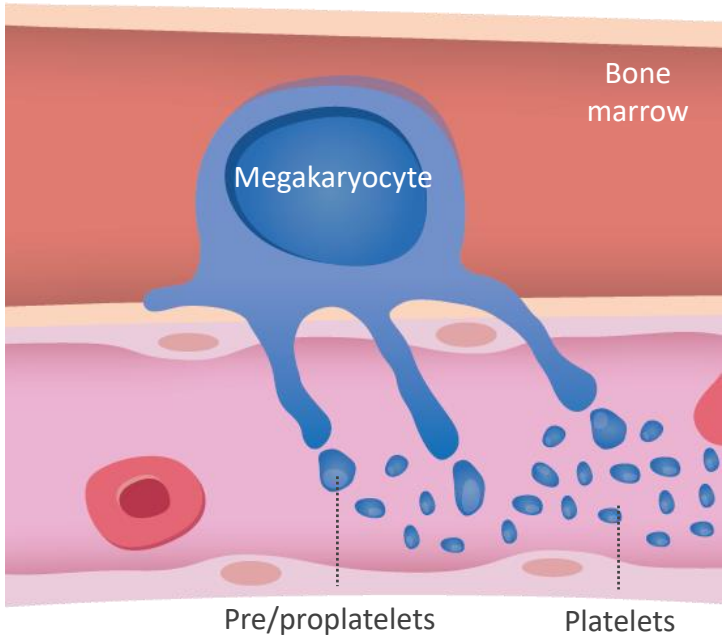
...ultimately resulting in the shedding of mature platelets



Adapted from French, 2013, Blood

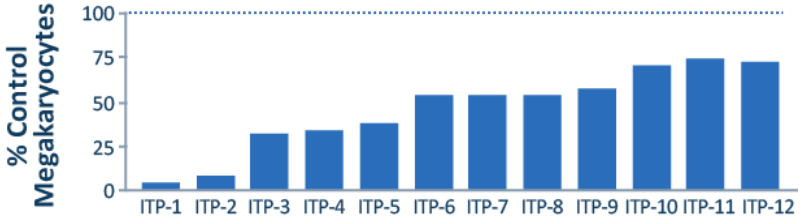
MoA 2: Autoantibodies Inhibit Platelet Production in ITP

Platelet glycoproteins are important mediators in megakaryocyte maturation and platelet production...

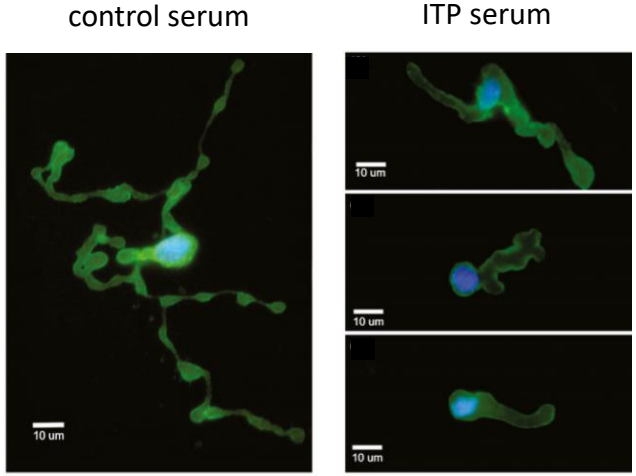


...explaining why megakaryocyte maturation and platelet formation are inhibited in ITP patients

Inhibition of megakaryocyte formation by ITP patient plasma[1]



Inhibition of proplatelet formation[2]

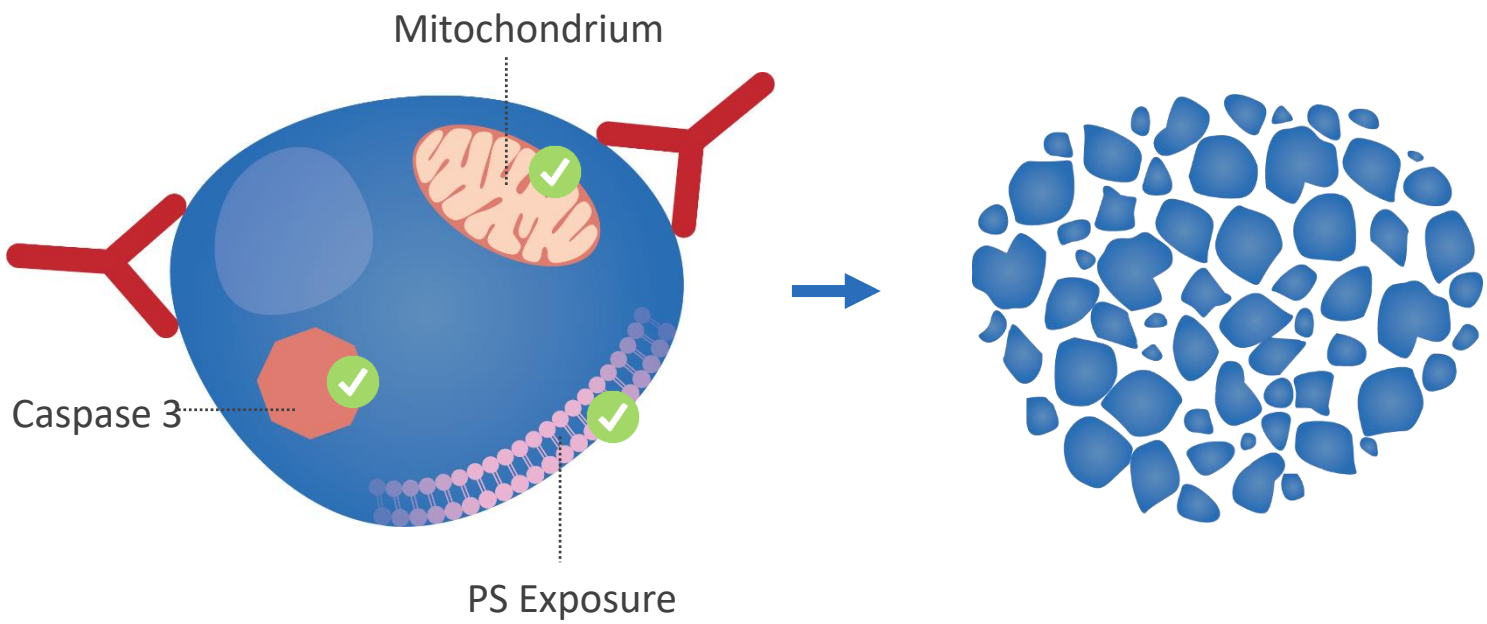


(1) McMillan, 2004, Blood, 2004; (2) Lev, 2014, Brit. Journ. Of Haematol.

MoA 3: Autoantibodies Induce Platelet Killing

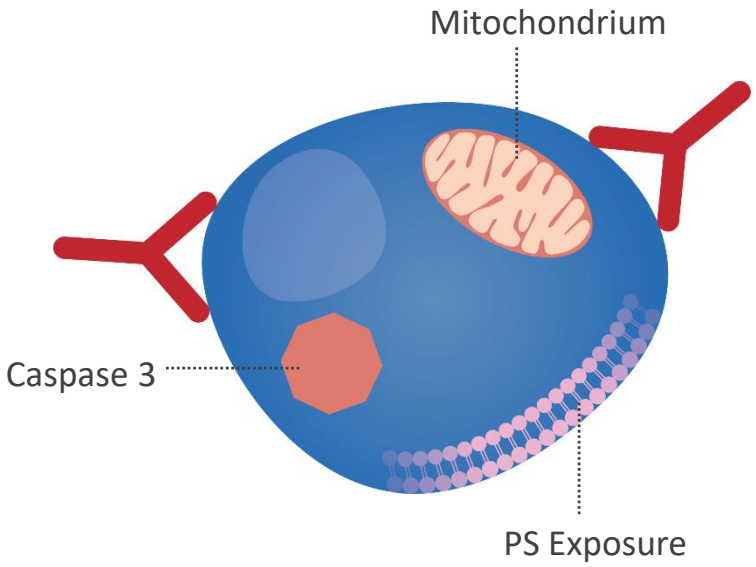
Platelets apoptosis marked by:

- loss of mitochondrial inner membrane potential (ΔmΨ)
- activation of caspase 3
- posphatidylserine (PS) externalization

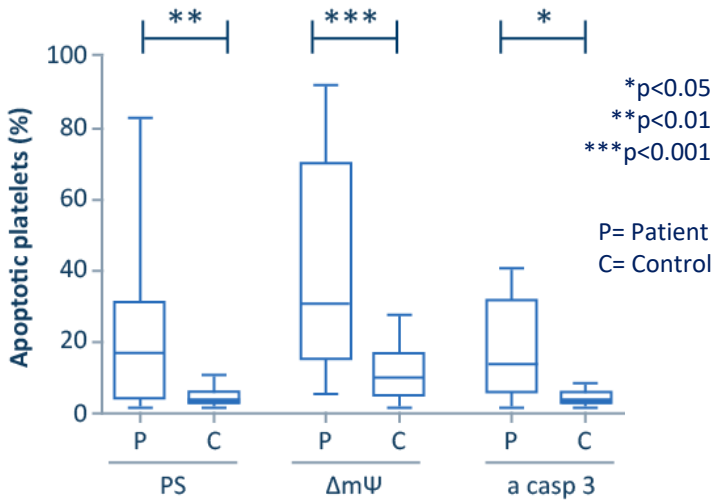


MoA 3: Autoantibodies Induce Platelet Killing in ITP

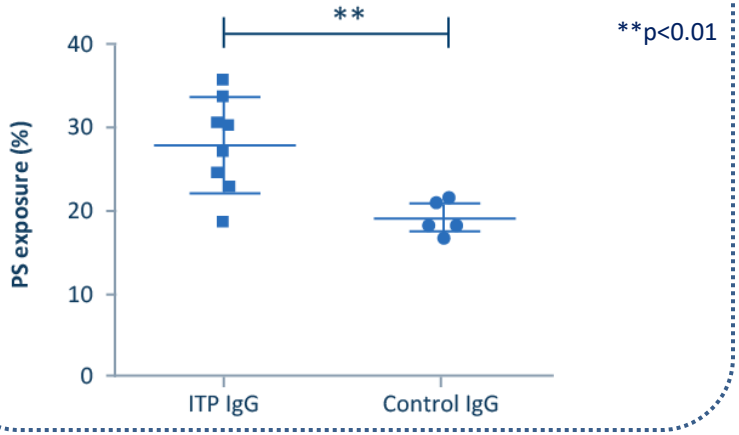
Autoantibodies induce platelet apoptosis[1] and can recruit complement[2]



ITP platelets display apoptotic signature….



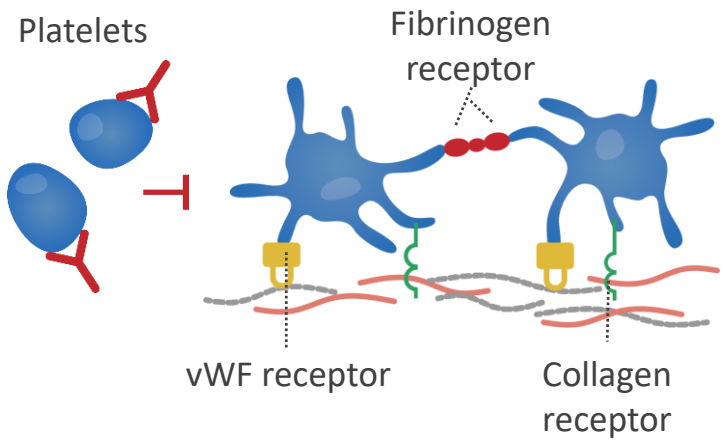
…. induced by autoantibodies



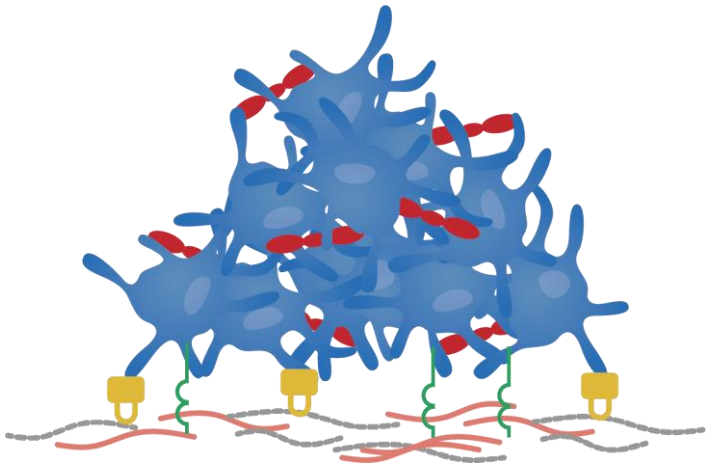
(1) Goette, 2016, PLOS; (2) Najaoui, 2011, Europ. Journ. of Haematol.

MoA 4: Autoantibodies Impair Platelet Function

Platelet adhesion and activation driven by interaction of platelet glycoprotein with collagen and vWF

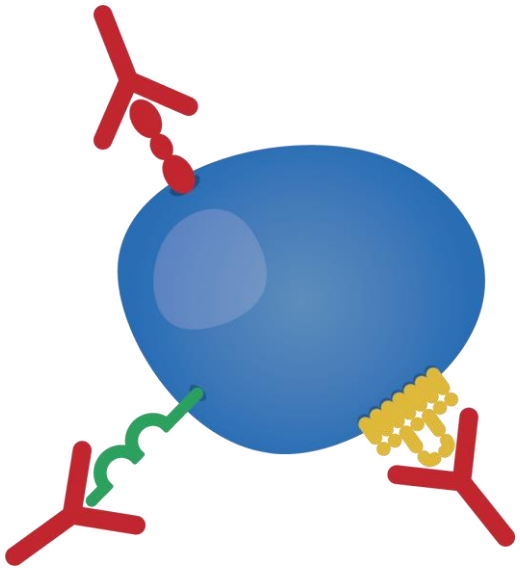


Platelet activation results in conformational changes, platelet-platelet interaction and thrombus formation

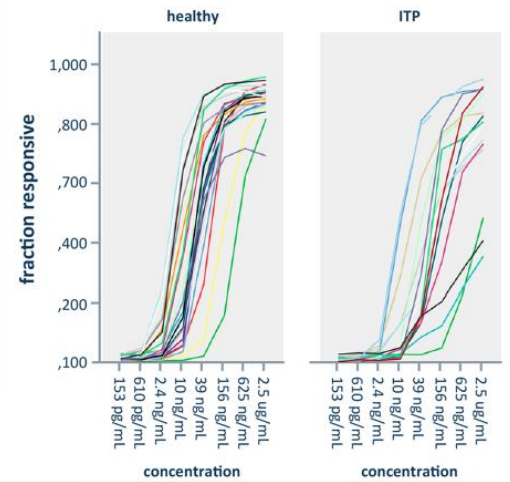


MoA 4: Autoantibodies Impair Platelet Function in ITP

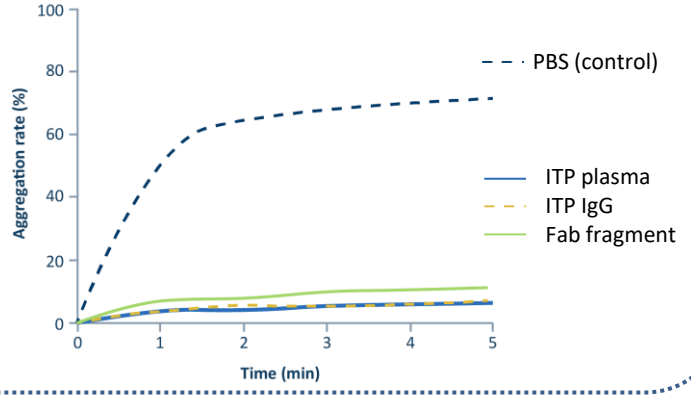
Autoantibodies interact with glycoproteins involved in platelet adhesion, activation and aggregation



Patients have aberrant platelet activation upon stimulation[1]



IgG of ITP patients inhibit platelet aggregation[2]

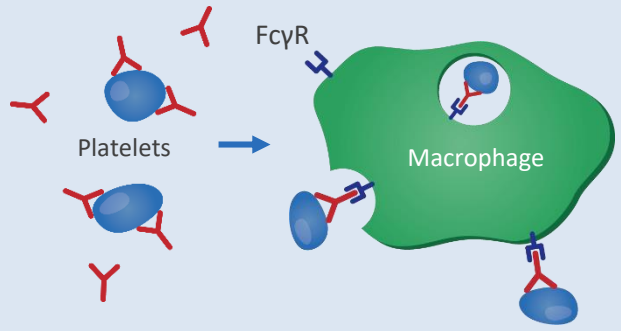


(1) Middelburg, 2016, Hematology; (2) Chu, 2006, Europ. Journ. Of Haematology

Efgartigimod Targets all Pathogenic AutoAb Actions Simultaneously

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

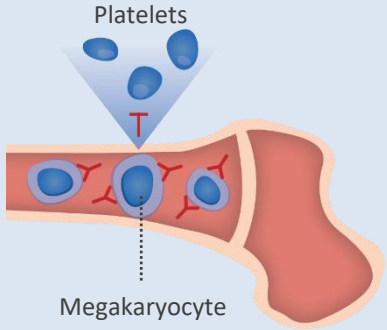
1. Accelerate platelet clearance



IVIg Tavalisse Splenectomy

Efgartigimod

2. Inhibit platelet production

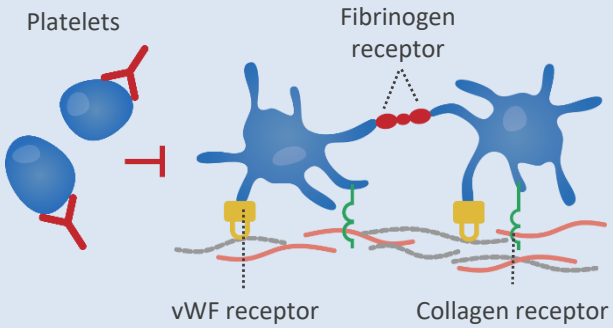


TPO-RA

Efgartigimod

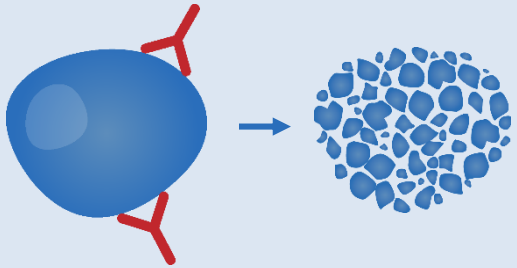
Autoantibodies

4. Interfere with platelet function



Efgartigimod

3. Induce platelet killing

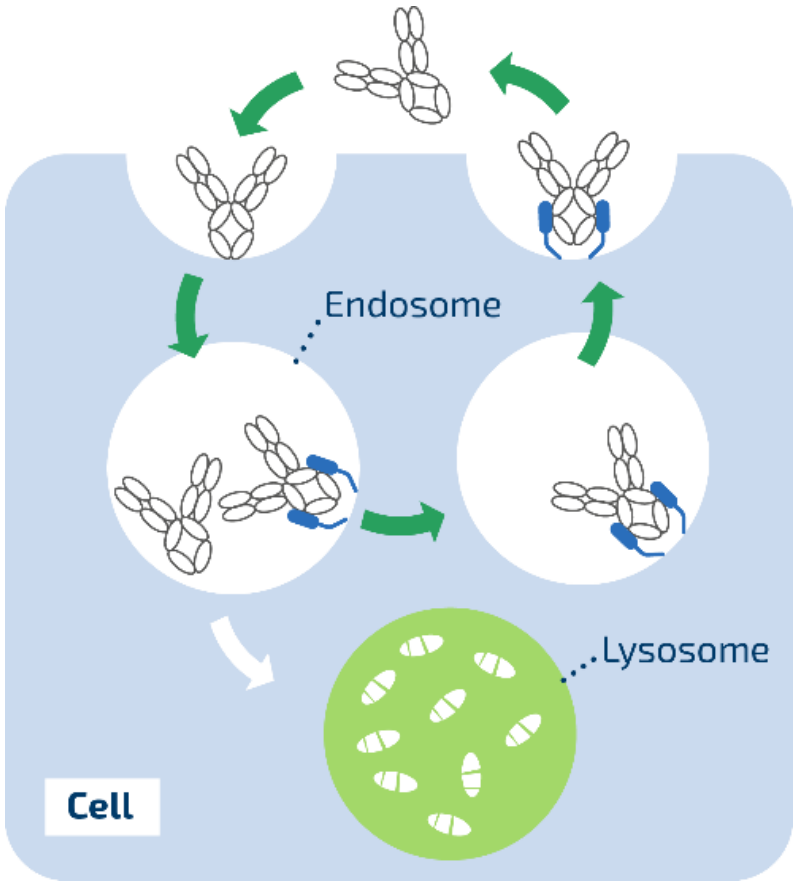


Efgartigimod

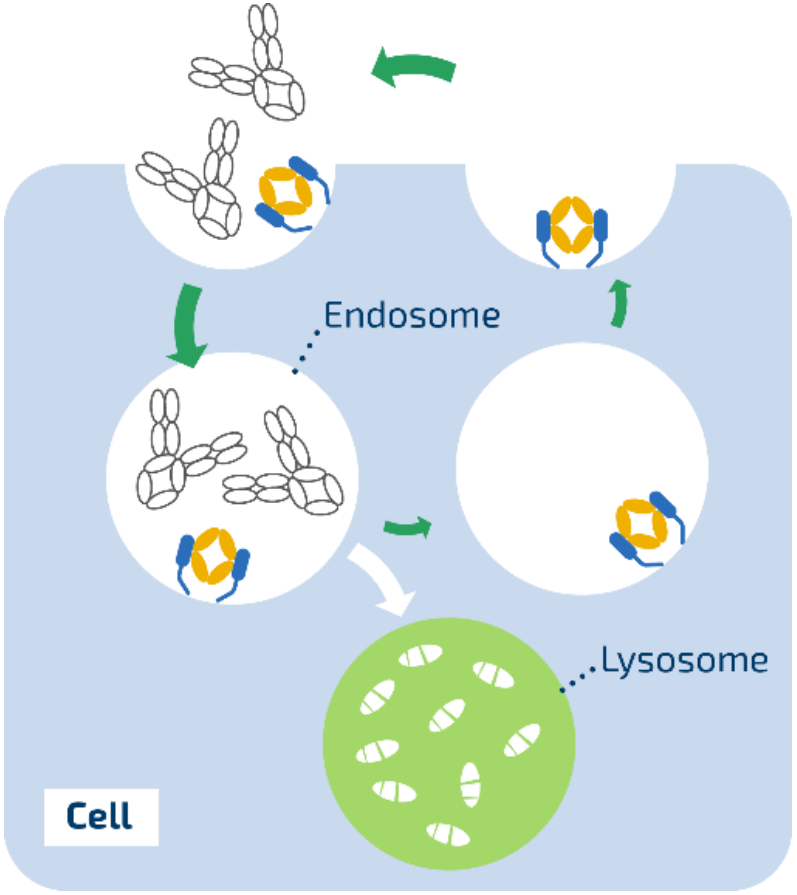
Efgartigimod: Lead Program Based on Novel Target FcRn

An innovative approach to eliminate IgG autoantibodies

IgG antibodies recycle through FcRn…



…Efgartigimod blocks FcRn leading to IgG elimination

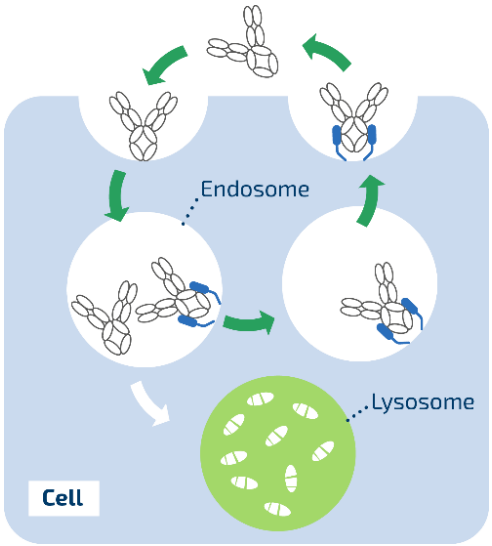


Antibody Efgartigimod FcRn

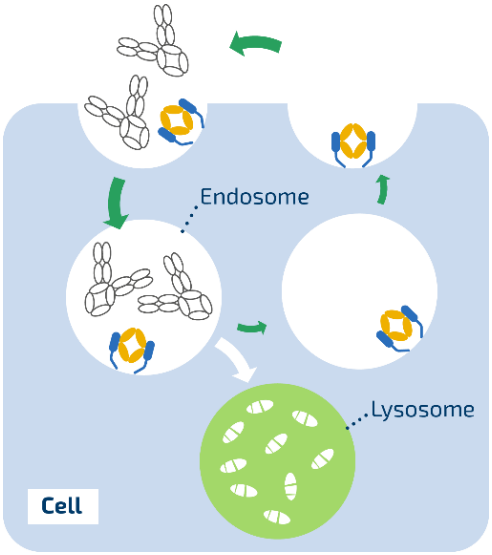
Efgartigimod: Lead Program Based on Novel Target FcRn

An innovative approach to eliminate IgG autoantibodies

IgG antibodies recycle through FcRn…



…Efgartigimod blocks FcRn leading to IgG elimination

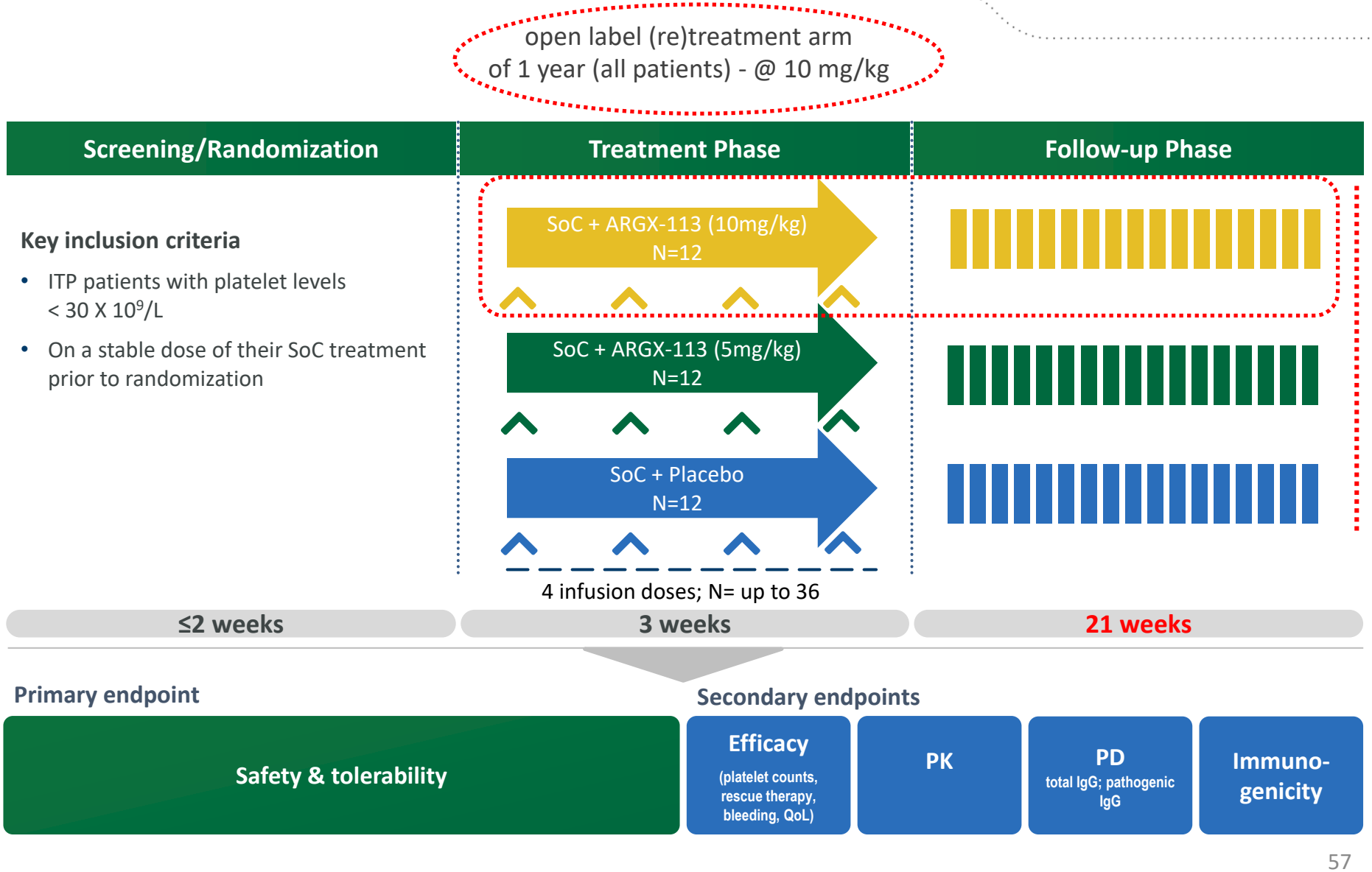


- Efgartigimod is a human IgG1 Fc-fragment that utilizes ABDEG™ Fc engineering technology[(2)(3)]
- Efgartigimod targets and binds to FcRn blocking the recycling of IgG leading to an elimination of IgG antibodies
- Demonstrated 50% to 85% reduction of circulating IgG antibody levels in Phase 1 trial
- Pathogenic IgG antibodies mediate multiple autoimmune diseases
- There is a correlation between disease severity and autoantibody levels in immune thrombocytopenia

(1) Roopenian et al. 2007, Nat Rev Immunol.; (2) Vaccaro et al. 2005, Nat Biotech. ; (3) argenx data

Efgartigimod: Phase 2 clinical trial in immune thrombocytopenia

Immune Thrombocytopenia Phase 2 Amended Trial Design



Efgartigimod:
A pipeline-in-a-product opportunity

Efgartigimod: A Pipeline-in-Product Opportunity

Prioritizing IgG autoantibody mediated diseases

Landscape of IgG severe auto-immune diseases (selection)

Immune Thrombocytopenia
Scleroderma
Lupus
Epidermolysis Bullosa Acquisita
Multiple Sclerosis
Anca Vasculitis
Myasthenia Gravis
Rheumatoid Arthritis
Pemphigus
Bullous Pemphigoid

Solid Biological Rationale
Disease proven to be predominantly mediated by pathogenics IgGs

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

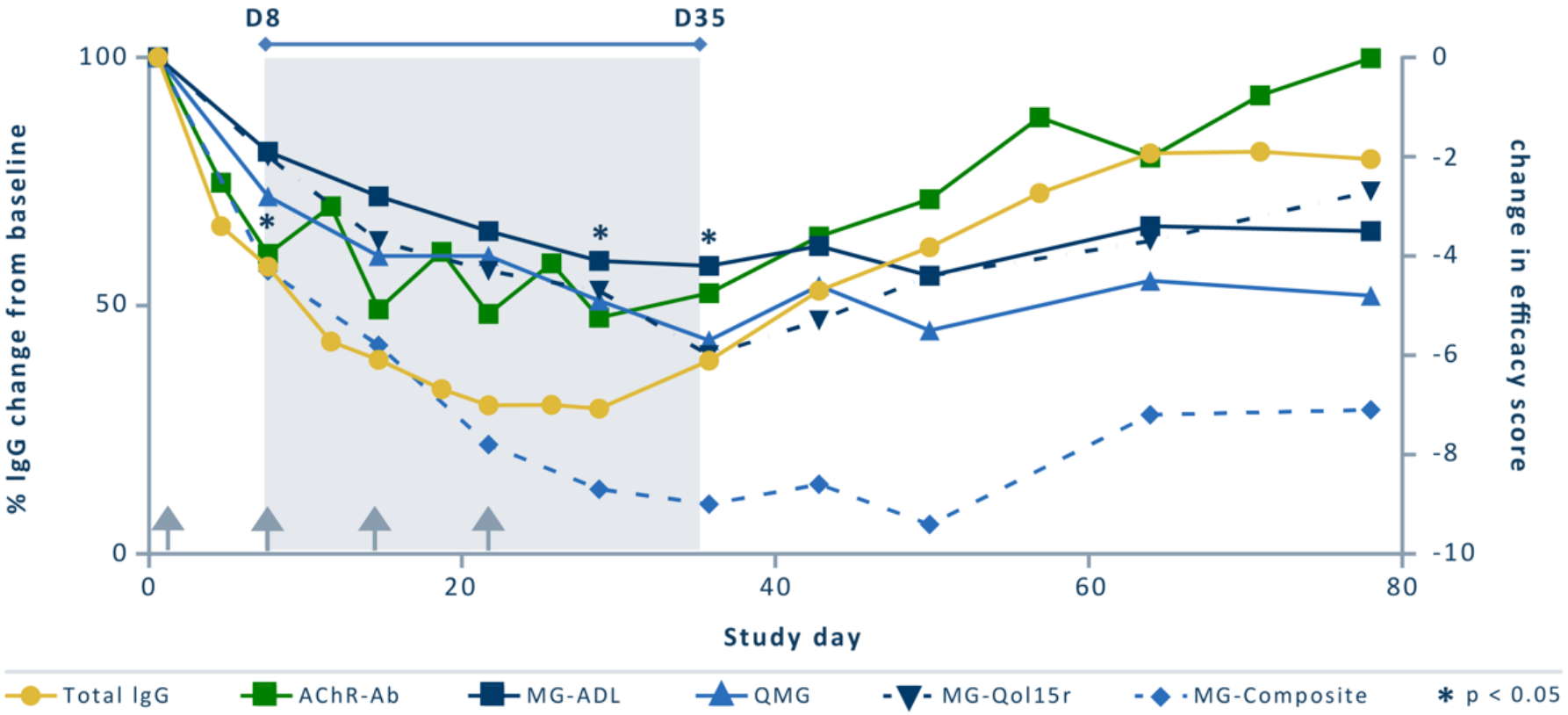
Proof of Concept Value
Spill-over effect into adjacent indications

Myasthenia Gravis	Immune Thrombocytopenia	Pemphigus Vulgaris
Beachhead neuromuscular diseases	Beachhead Heme disorders	Beachhead blistering diseases

argenx data

Total & Pathogenic IgG Reduction Correlates with Clinical Improvements

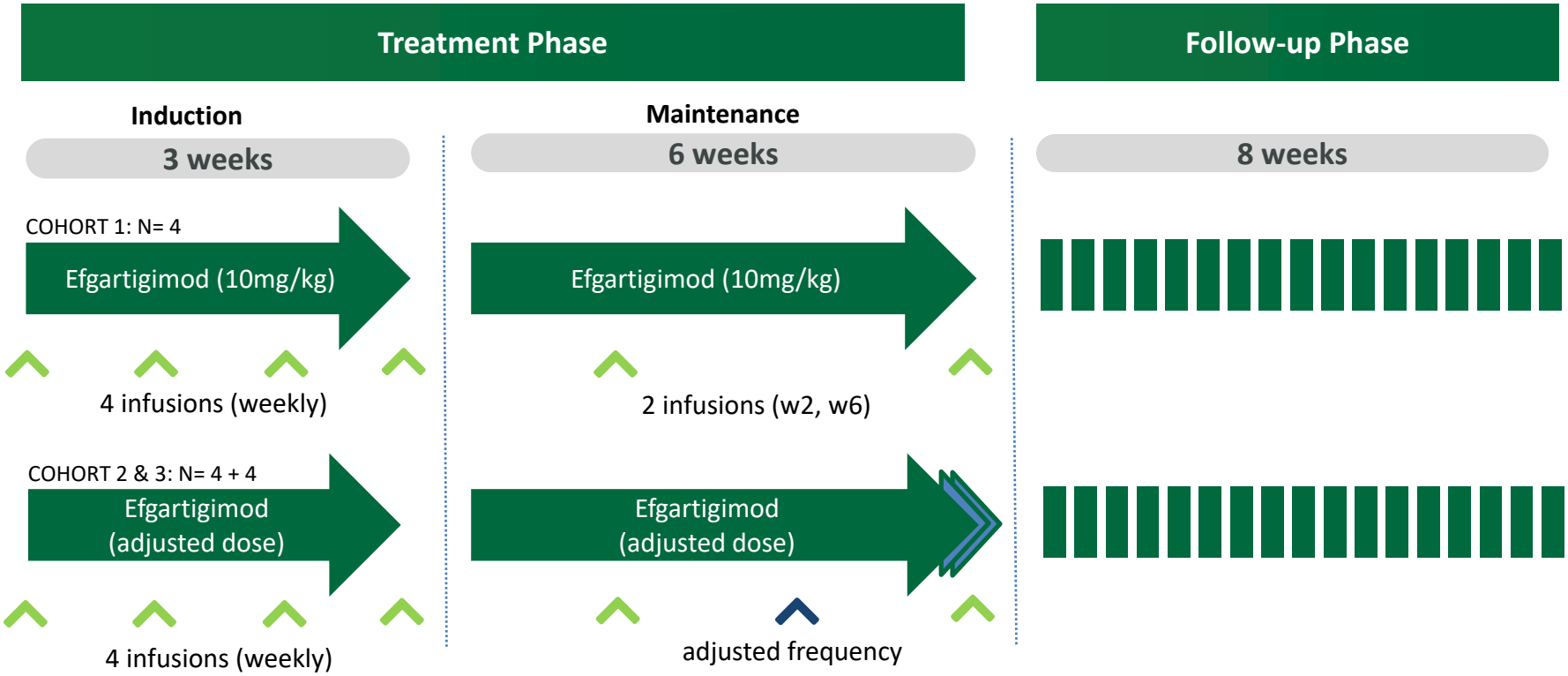
Assessment for all efficacy scales



- Clinical improvement persists despite return of IgG levels
- Potential differentiation from PLEX, where clinical benefit was reported to be lost 2-4 weeks after end of treatment [1]

(1) Kuks and Skallebaek, 1998, Transfus Sci

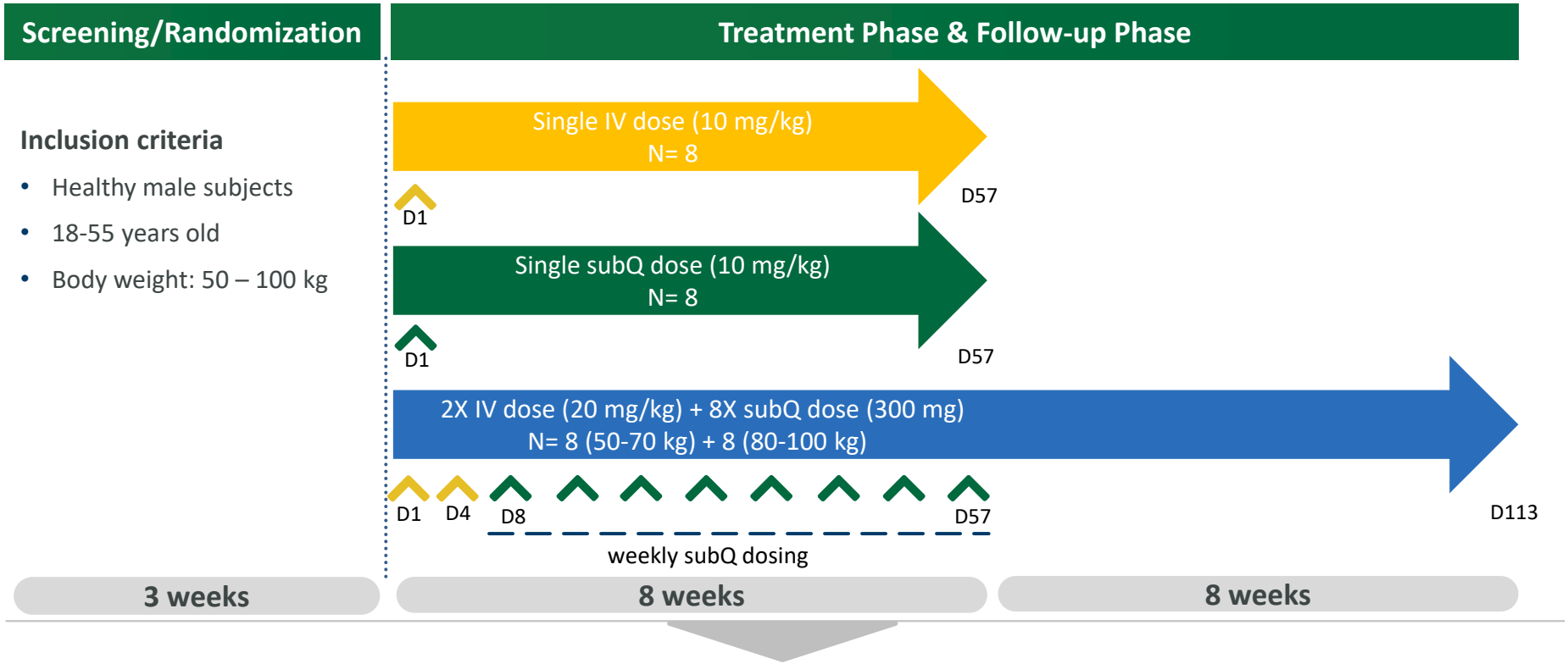
Pemphigus Vulgaris Phase 2 Adaptive Design



- Cohort 1: 10 mg/kg, induction = 4 infusions (3 weeks), maintenance = 2 infusions (6 weeks)
- Additional cohorts:
  - Dose up (25mg/kg) or down
  - Change frequency of dosing at maintenance
  - Extend maintenance duration

Phase 1 Healthy Volunteer SubQ Formulation

Open Label Trial Design



Read out

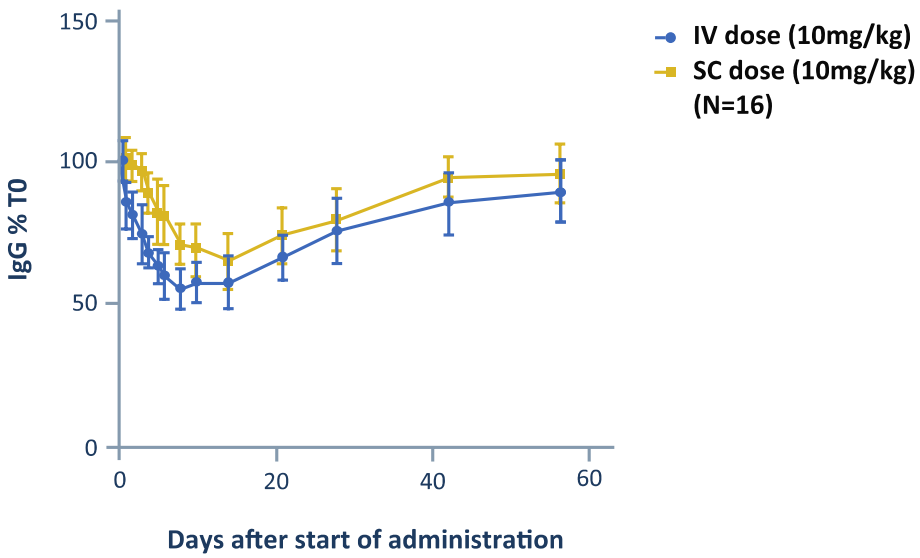
Safety & tolerability	PK	PD Total IgG; IgG subtypes; IgA & IgM	Immunogenicity

Clinicaltrials.gov: NCT03334084, argenx data

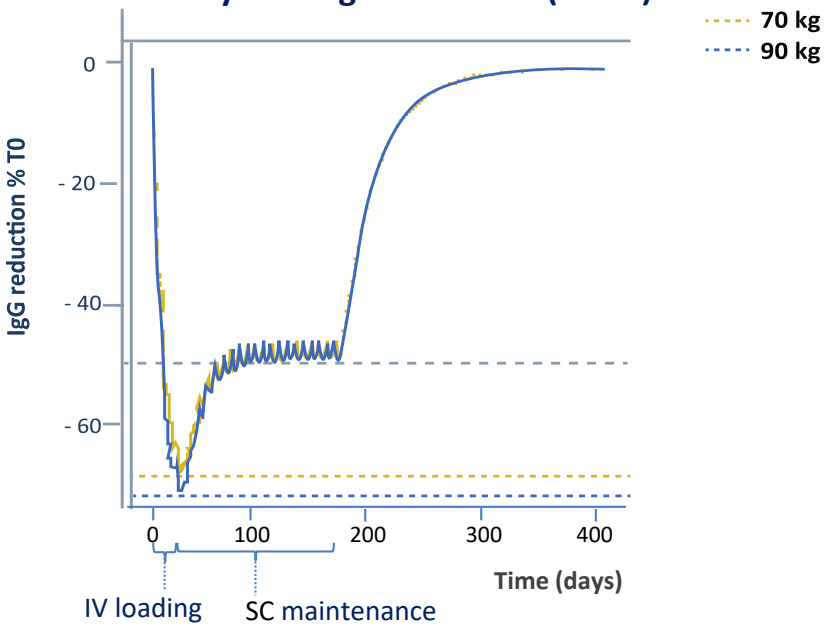
Efgartigimod: Feasibility of Subcutaneous Dosing

Providing optionality to patients

Comparable IgG reduction



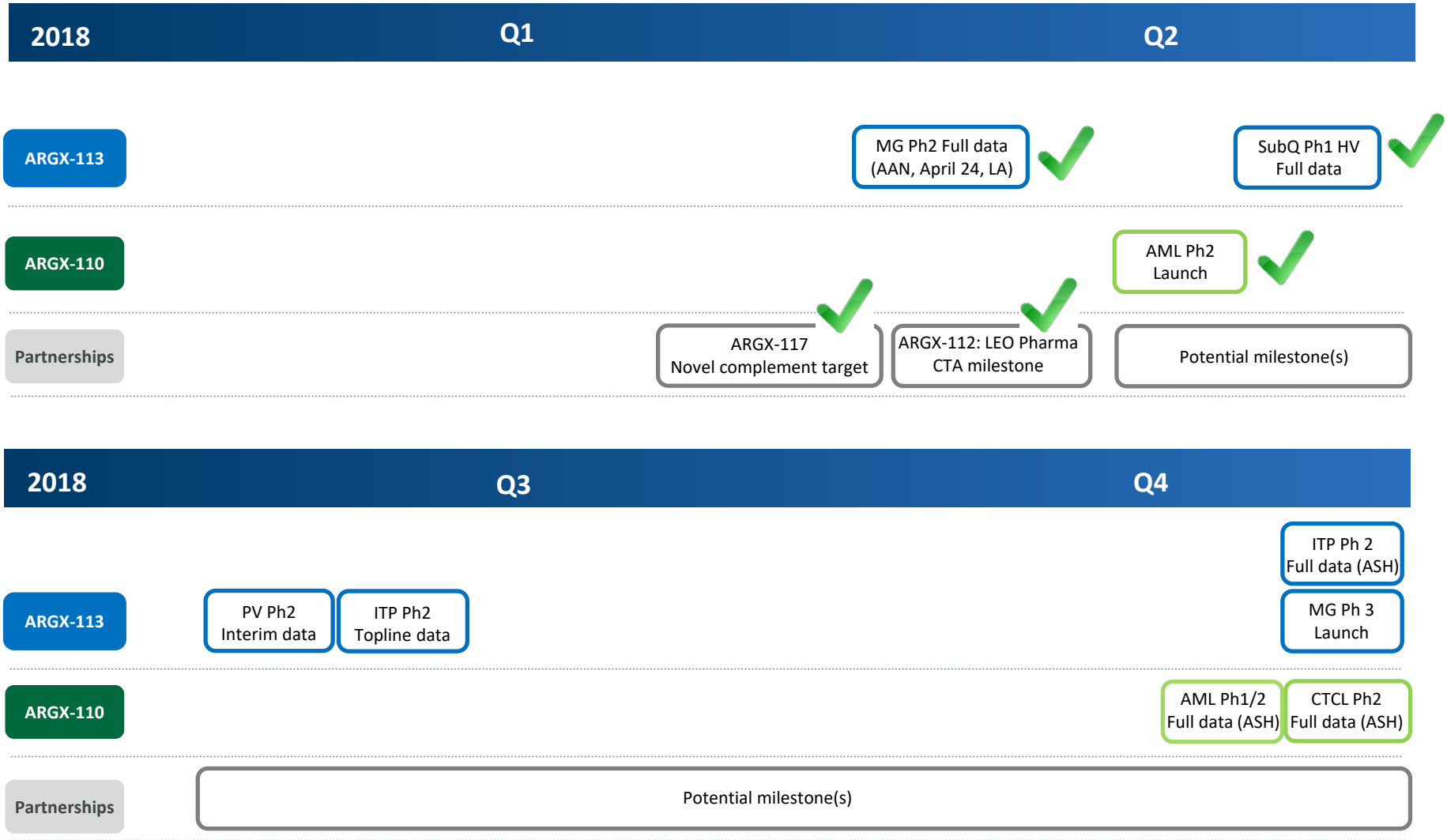
Steady state IgG reduction (~50%)



- Viability of SC formulation demonstrated:
  - Comparable half-life to IV
  - Comparable IgG reduction to IV; steady state 50% IgG reduction achieved by weekly dosing (300 mg fixed dose)
  - Favorable bio-availability (~ 50%)
  - Favorable viscosity and stability profile

argenx uncleaned data

Key Upcoming Expected Milestones & Communications



Agenda

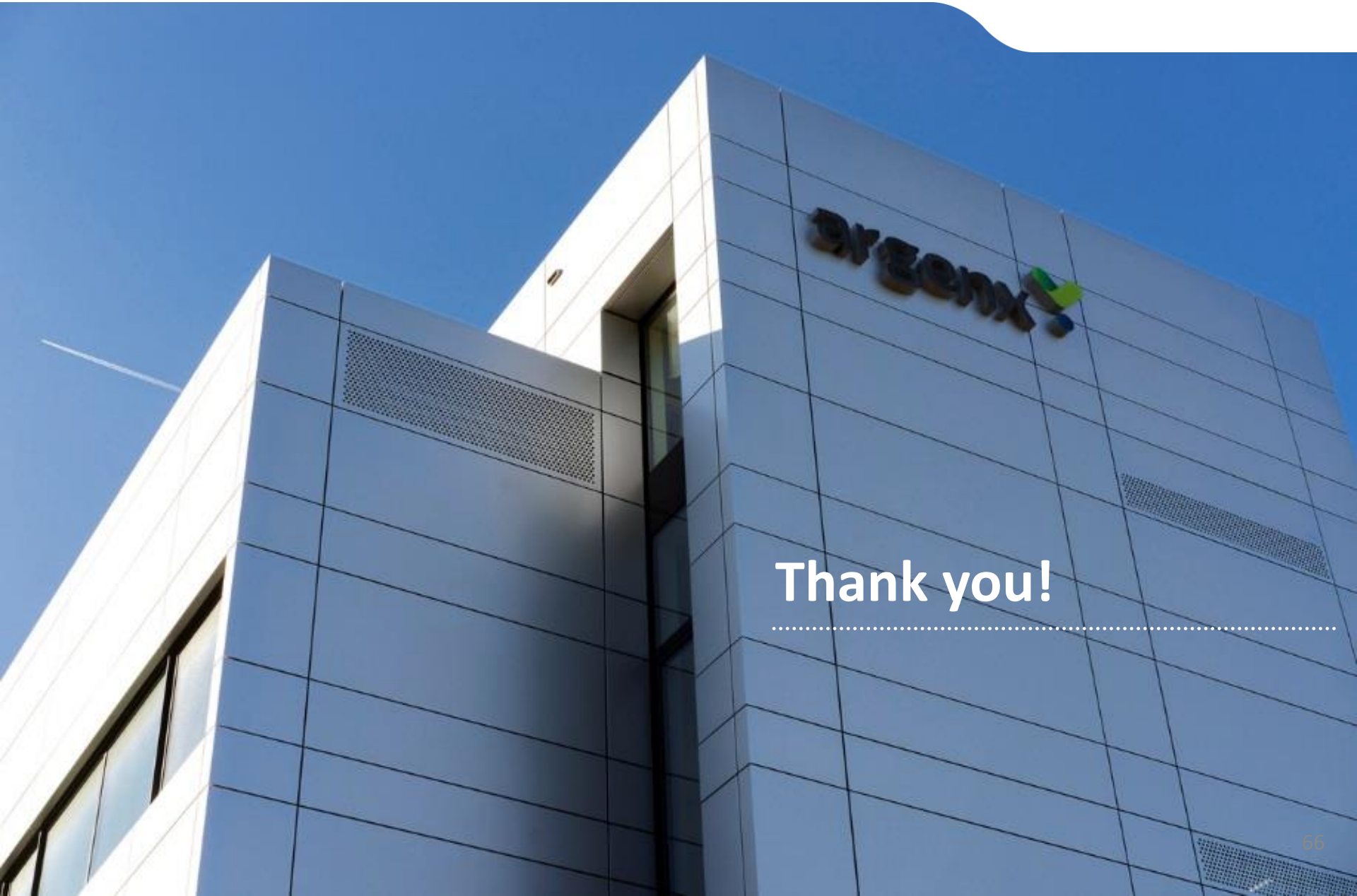
08:30 **Welcome & Introduction**
Tim Van Hauwermeiren, argenx

08:35 **Efgartigimod: Phase 2 Study in immune thrombocytopenia**

- **Introduction to immune thrombocytopenia**
  Catherine Broome, MD, Georgetown University, Washington, DC
- **Attractive market opportunity**
  Rebecca Rupert, argenx
- **Strong biologic rationale**
  Peter Ulrichts, PhD, argenx
- **Phase 2 clinical trial in immune thrombocytopenia**
  Tim Van Hauwermeiren, argenx

09:35 **Efgartigimod: Pipeline-in-product opportunity**
Tim Van Hauwermeiren, argenx

09:45 **Q&A**



Thank you!