Meeting the demand of PDMPs

Albert Farrugia

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University of Western Australia

Scientific Consultant

International Plasma and Fractionation Association

ALL OPINIONS VOICED IN THIS PRESENTATION ARE STRICTLY MINE, AND DO NOT REPRESENT THOSE OF ANY OF MY AFFILIATIONS

….ALTHOUGH I KEEP WORKING ON IT.

The concept of Latent Therapeutic Demand

- *Usage* approaches *sufficiency* as supply approaches the *latent therapeutic demand*
- We define Latent Therapeutic Demand (LTD) as *the underlying demand that represents how physicians would prescribe treatment and how patients would comply with the prescribed treatment if ample supplies were available and affordable, and access to therapy was unencumbered by issues other than evidence-based clinical need, such as financial constraints.*

Estimating latent therapeutic demand plasma therapies

J Clin Immunol (2014) 34:233–244
DOI 10.1007/s10875-013-9975-1

ORIGINAL RESEARCH

Modeling Primary Immunodeficiency Disease Epidemiology and Its Treatment to Estimate Latent Therapeutic Demand for Immunoglobulin

Jeffrey S. Stonebraker · Albert Farrugia · Benjamin Gathmann · ESID Registry Working Party · Jordan S. Orange

Received: 6 January 2021 | Revised: 3 May 2021 | Accepted: 4 May 2021
DOI: 10.1111/vox.13134

ORIGINAL ARTICLE

Vox Sanguinis ISBT International Society of Blood Transfusion

Estimation of the latent therapeutic demand for immunoglobulin therapies in autoimmune neuropathies in the United States

Albert Farrugia[1] | Megha Bansal[2] | Ivan Marjanovic[3]

Vox Sanguinis (2018) **113**, 430–440

ORIGINAL PAPER

DOI: 10.1111/vox.12651

Latent therapeutic demand model for the immunoglobulin replacement therapy of primary immune deficiency disorders in the USA

J. S. Stonebraker,[1] J. Hajjar[2] & J. S. Orange[2]
[1]Department of Business Management, Poole College of Management, North Carolina State University, Raleigh, NC, USA
[2]Section of Immunology, Allergy and Rheumatology, Texas Children's Hospital, Department of Pediatrics, Baylor College of Medicine, Houston, TX, USA

Haemophilia (2004), **10**, 18–26

Modelling haemophilia epidemiology and treatment modalities to estimate the unconstrained factor VIII demand

J. S. STONEBRAKER,* R. E. AMAND,* M. V. BAUMAN,* A. J. NAGLE† and P. J. LARSON*
*Bayer HealthCare, Biological Products Division, Research Triangle Park, NC, USA; †Formerly with Bayer Healthcare, now with Novozymes A/S, Bagsvaerd, Denmark

Critical Care BMC

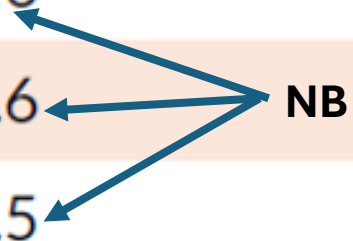
▸ Crit Care. 2015 Mar 16;19(Suppl 1):P354. doi: 10.1186/cc14434

Estimation of the latent therapeutic demand for albumin in the USA: a focus on three indications

A Farrugia [1], M Bansal [2]

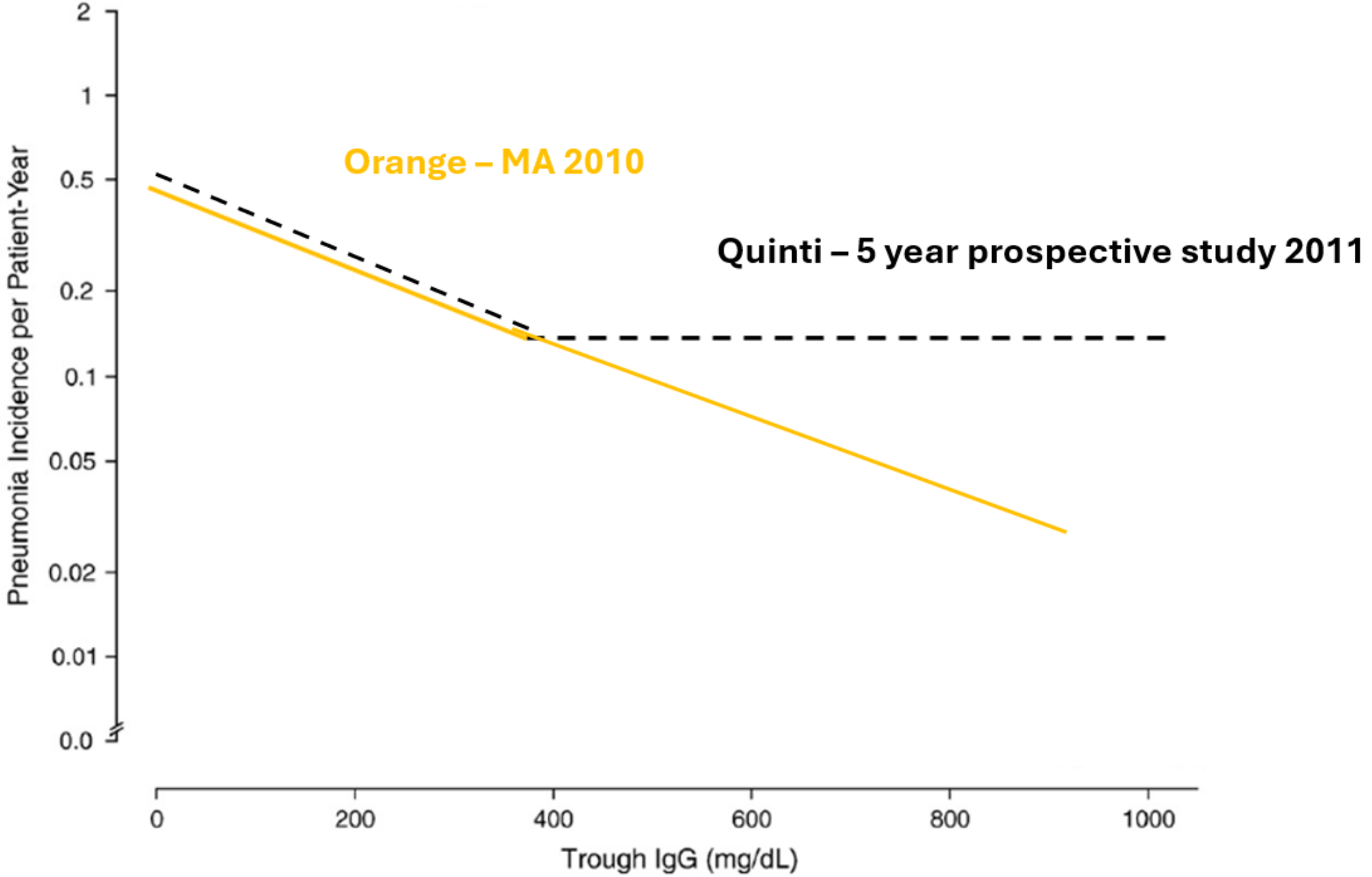
Latent therapeutic demand for IG in the USA
Primary Immune Deficiencies

Condition	Mean LTD immunoglobulin g/10^3 population
Common variable immune deficiency (CVID)	65.4 ± 73.6
X-linked agammaglobulinemia (XLA)	25.5 ± 27.6
Severe combined immune deficiency (SCID)	13.4 ± 13.5
Wiskott–Aldrich syndrome (WAS)	0.5 ± 0.4
hyper IGM syndrome (HIGM)	0.3 ± 0.3

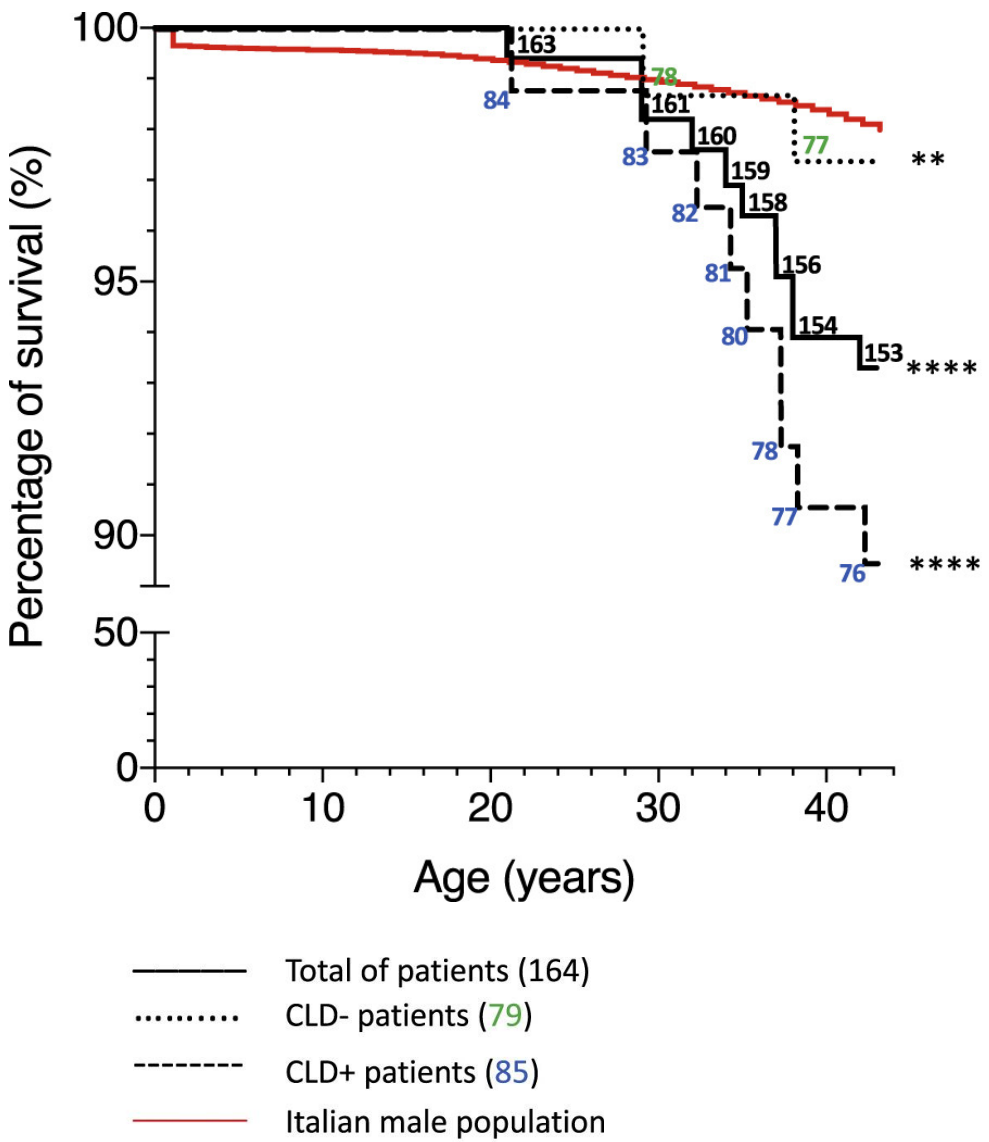


Vox Sang, 113: 430-440.

IG through levels and risk of pneumonia in PID



Survival of Italian patients with XLA during long-term follow-up

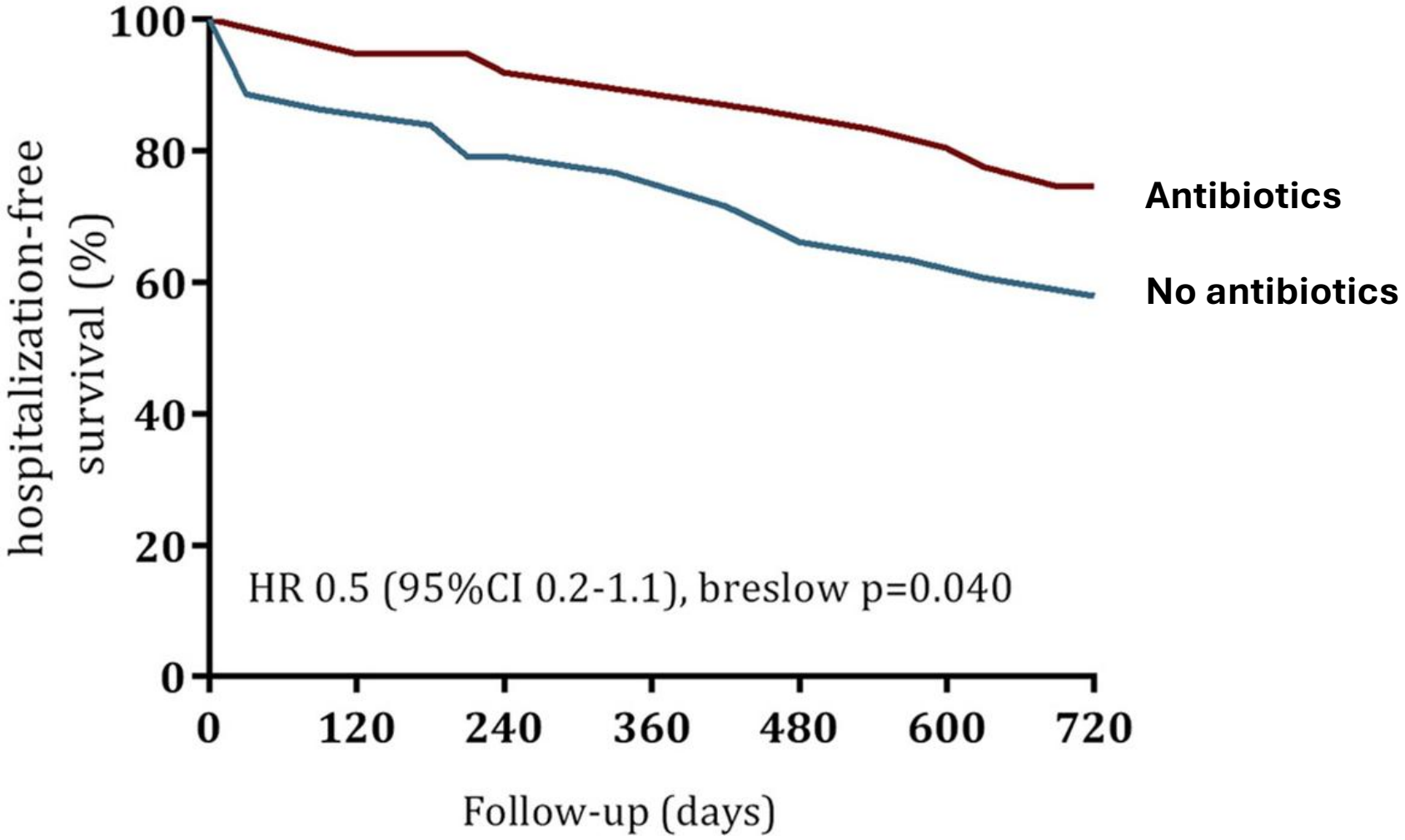


"The high prevalence of CLD in our cohort suggests that ***regular IgG supplementation does not prevent development of this complication****.... Considering the impact of CLD on everyday life and especially on long-term outcome, physicians should pay more attention to lung morbidity in XLA and* ***consider****, as early as possible, a personalized respiratory physiotherapy program and/or* ***an antibiotic prophylaxis regimen for affected patients****."*

Journal of Allergy and Clinical Immunology, 2020 Volume 146, Issue 2, 429 - 437

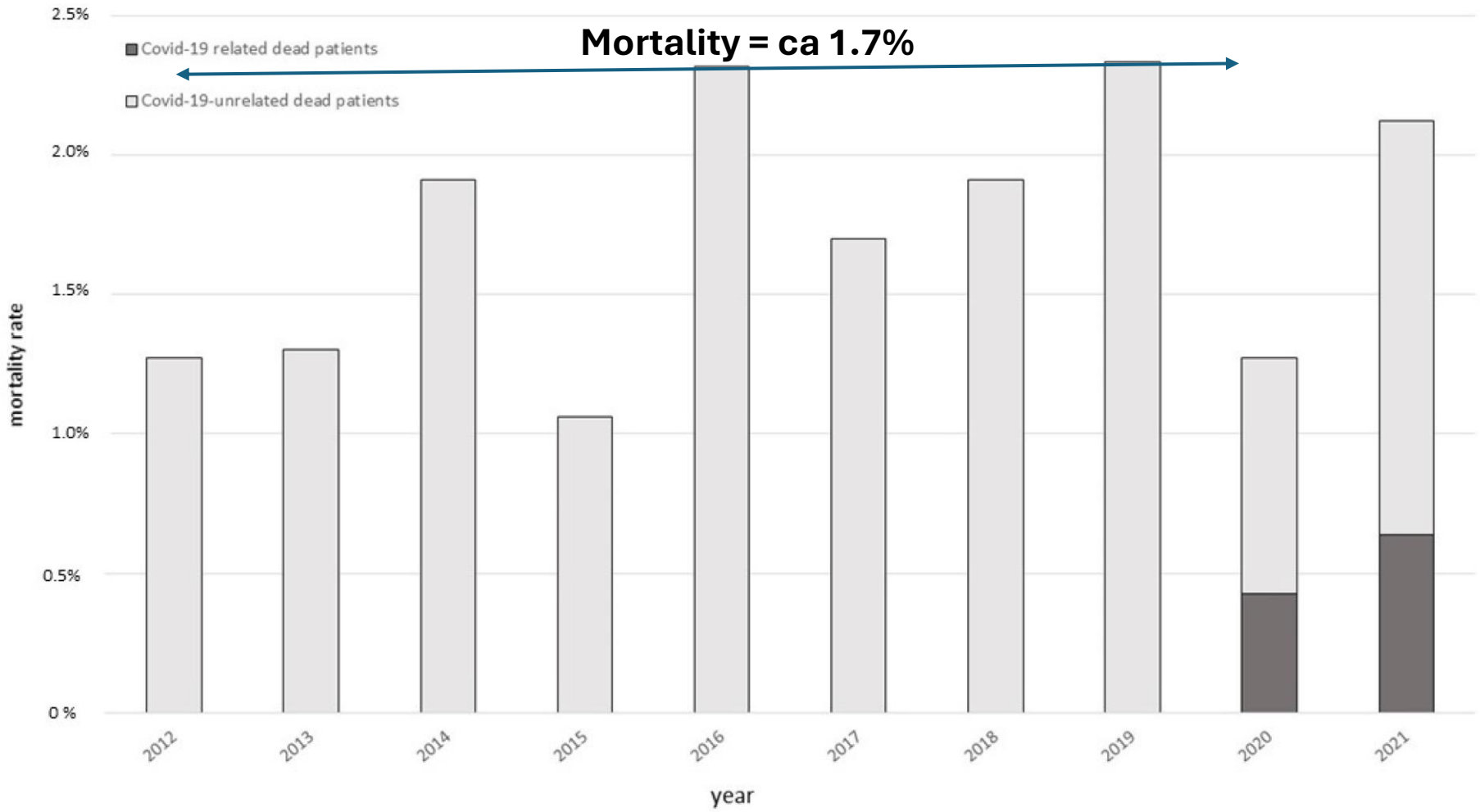
Double-blind, placebo-controlled, randomized trial on low-dose azithromycin prophylaxis in patients with primary antibody deficiencies

Patients on individualized IVIG



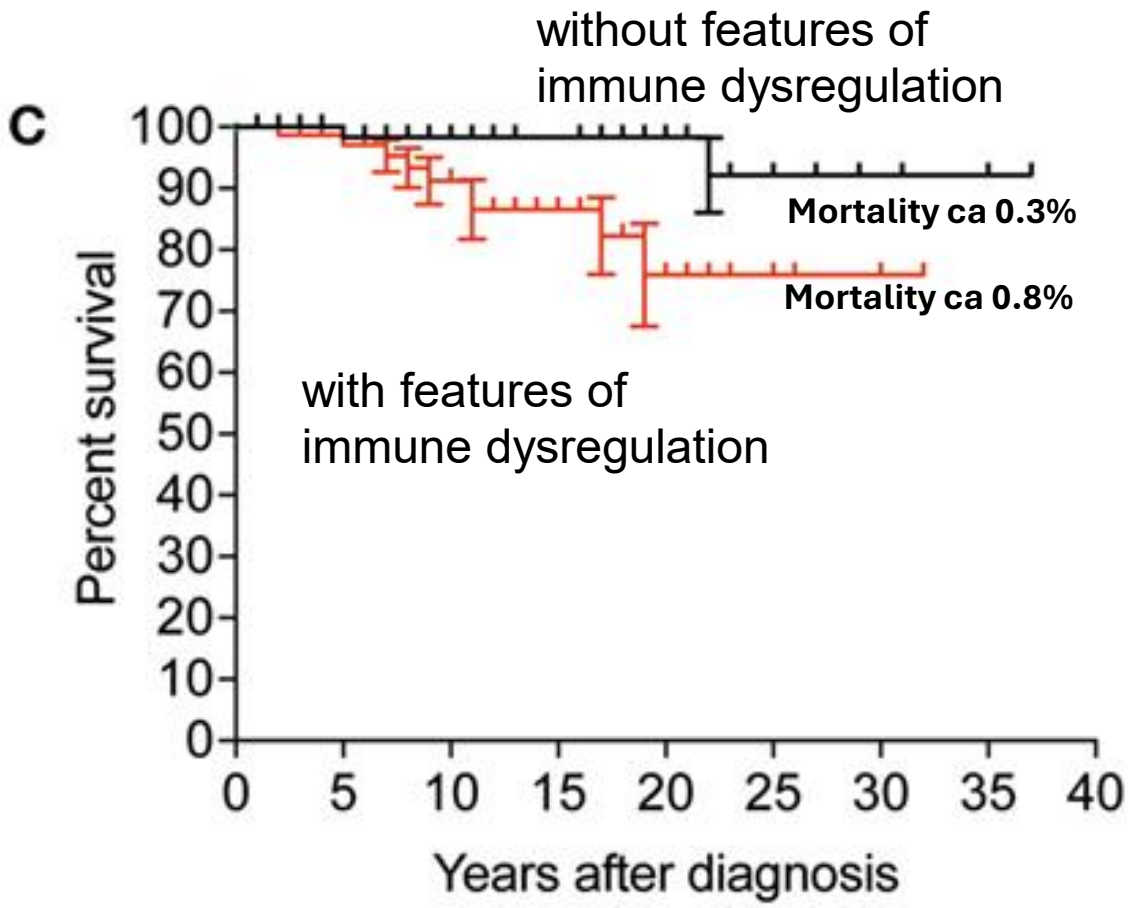
Journal of Allergy and Clinical Immunology 2019, Volume 144, Issue 2, 584 - 593.e7

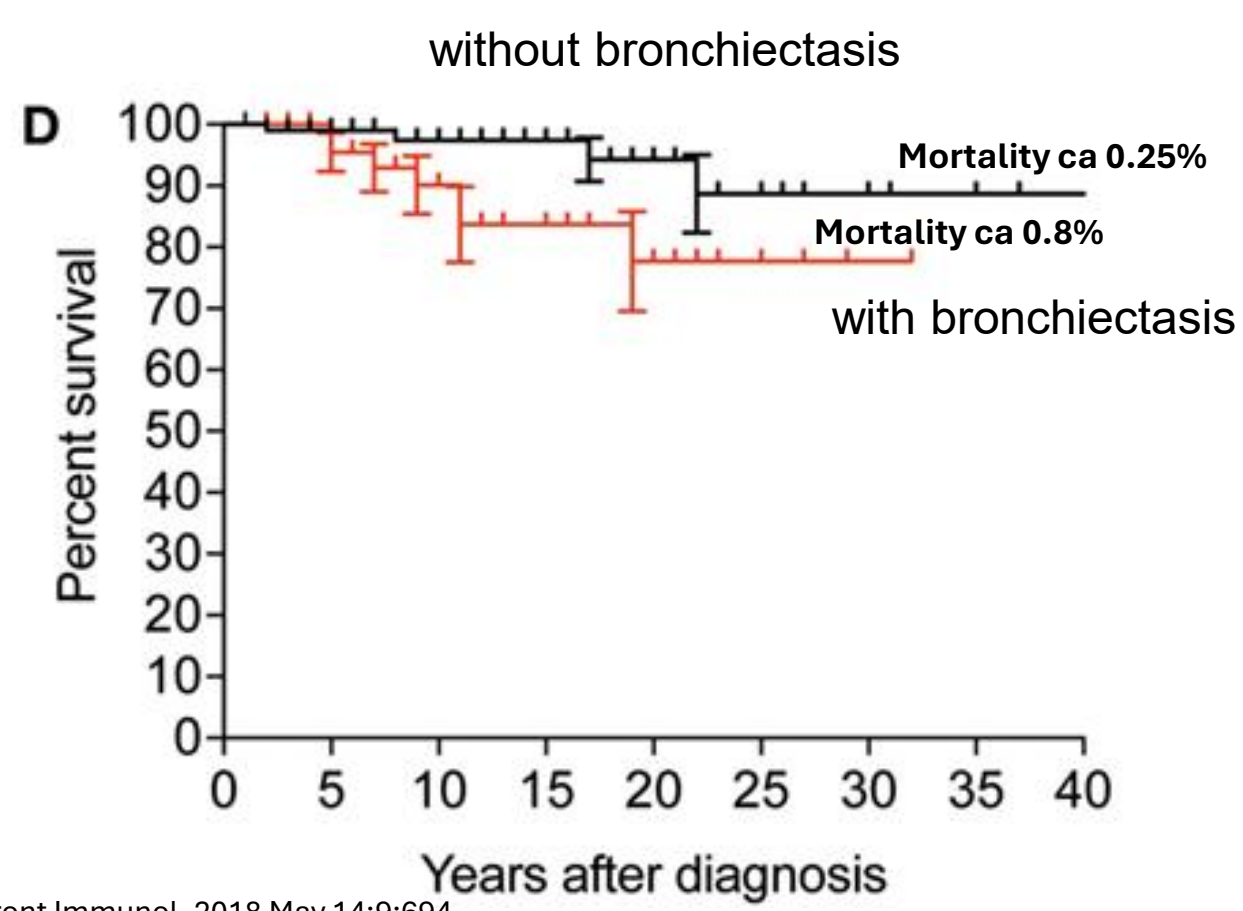
Mortality in Severe Antibody Deficiencient Italian Patients



Biomedicines 2022, 10, 1026. https://doi.org/10.3390/biomedicines10051026

Mortality in a cohort of Australian PID patients *Treated with IG*





Front Immunol. 2018 May 14:9:694

Bodies in the streets? –

I - In 2016 UK used > 1/3 of the IG/capita that Australia used – BUT.....

Clinical & Experimental Immunology
The Journal of Translational Immunology
British Society for immunology

Clinical and Experimental Immunology ORIGINAL ARTICLE doi:10.1111/cei.12748

Clinical and laboratory correlates of lung disease and cancer in adults with idiopathic hypogammaglobulinaemia

Four hundred and seventy-three (59%) patients suffered from overt bacterial LRTI and 377 (47%) from bronchiectasis (Table 1). Patients with a history of bacterial LRTI

Primary immunodeficiency disease: a cost-utility analysis comparing intravenous *vs* subcutaneous immunoglobulin replacement therapy in Australia

Tanja M. Windegger[1], Son Nghiem[2], Kim-Huong Nguyen[3,4], Yoke L. Fung[1], Paul A. Scuffham[2]

Therefore, side effects and adverse events were not included in the model.

In our cohort, 21% of patients had bronchiectasis when they joined the study. Of the PID patients with bronchiectasis, 50% went to hospital ED instead of the GP when seeking

- ***.....the data available for the rate of bronchiectasis in PID patients in the UK is twice that of PID patients in Australia.***
- ***Bronchiectasis is an indicator of***
  1. ***Diagnostic delay***
  2. ***Undertreatment***

Bodies in the streets?

II - NZ uses 1/3 of the IG/capita that Australia uses – BUT.....

Clinical and Experimental Immunology ORIGINAL ARTICLE doi: 10.1111/cei.13595

Bronchiectasis is associated with delayed diagnosis and adverse outcomes in the New Zealand Common Variable Immunodeficiency Disorders cohort study

‡Department of Molecular Medicine and Pathology, University of Auckland, §School of Population Health, University of Auckland, ¶Population Health Directorate, Counties Manukau Health, Auckland, **Department of Clinical Immunology, Fiona Stanley Hospital, Perth, WA Australia, ††Department of Clinical Immunology, Christchurch Hospital

as early-onset disease, delay in diagnosis and increased numbers of infections were associated with greater risk of bronchiectasis. One hundred and seven adult patients with a diagnosis of CVID are currently enrolled in the NZCS, comprising approximately 70% of patients known to have CVID in New Zealand. Fifty patients (46·7%) had radiologically proven bronchiectasis. This study has shown that patients with compared to those without bronchiectasis have an increased mortality at a younger age. CVID patients with bronchiectasis had a greater number of severe infections consequent

Primary immunodeficiency disease: a cost-utility analysis comparing intravenous *vs* subcutaneous immunoglobulin replacement therapy in Australia

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  1. ***Diagnostic delay***
  2. ***Undertreatment***

IPOPI Global Survey – HRQoL in PID

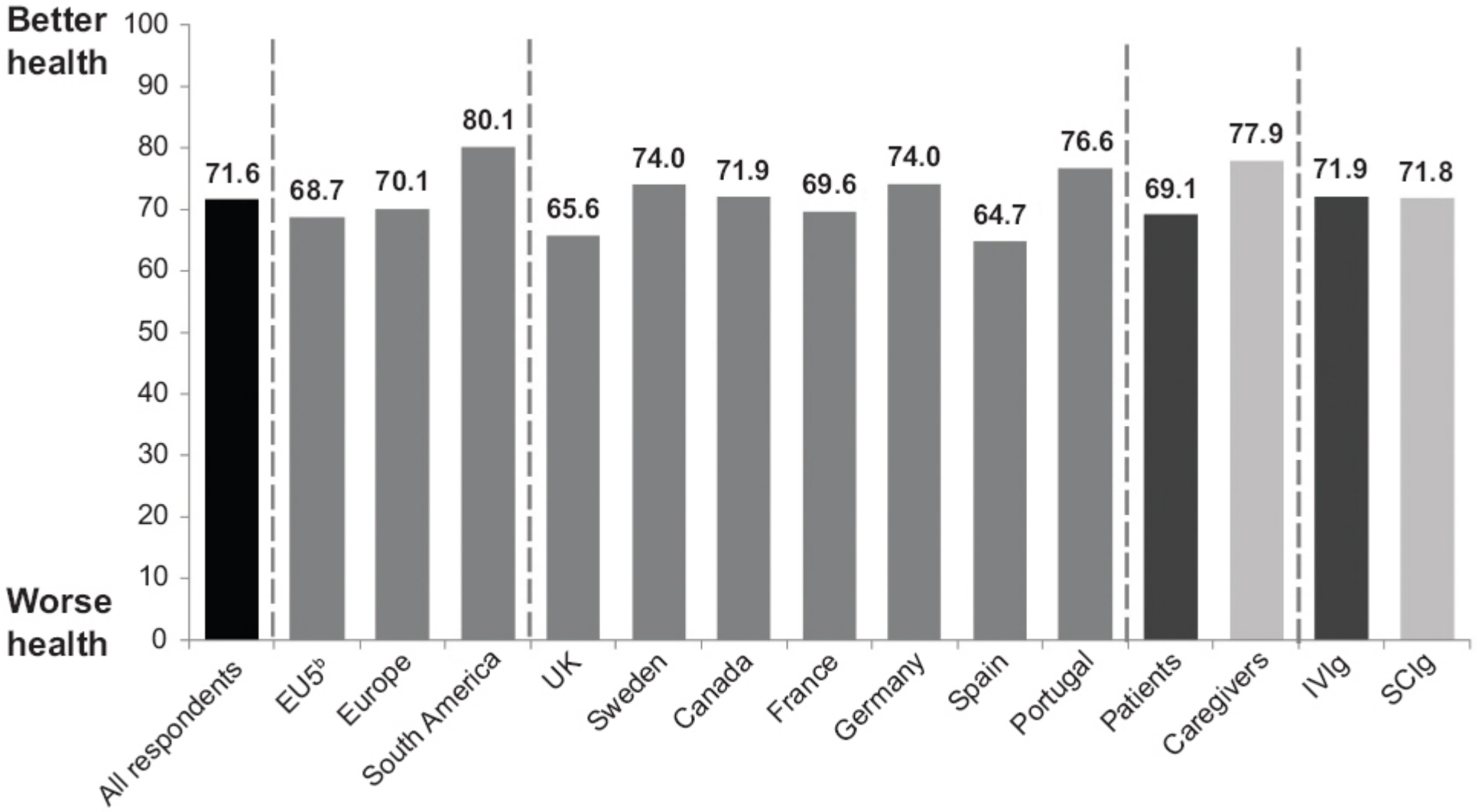
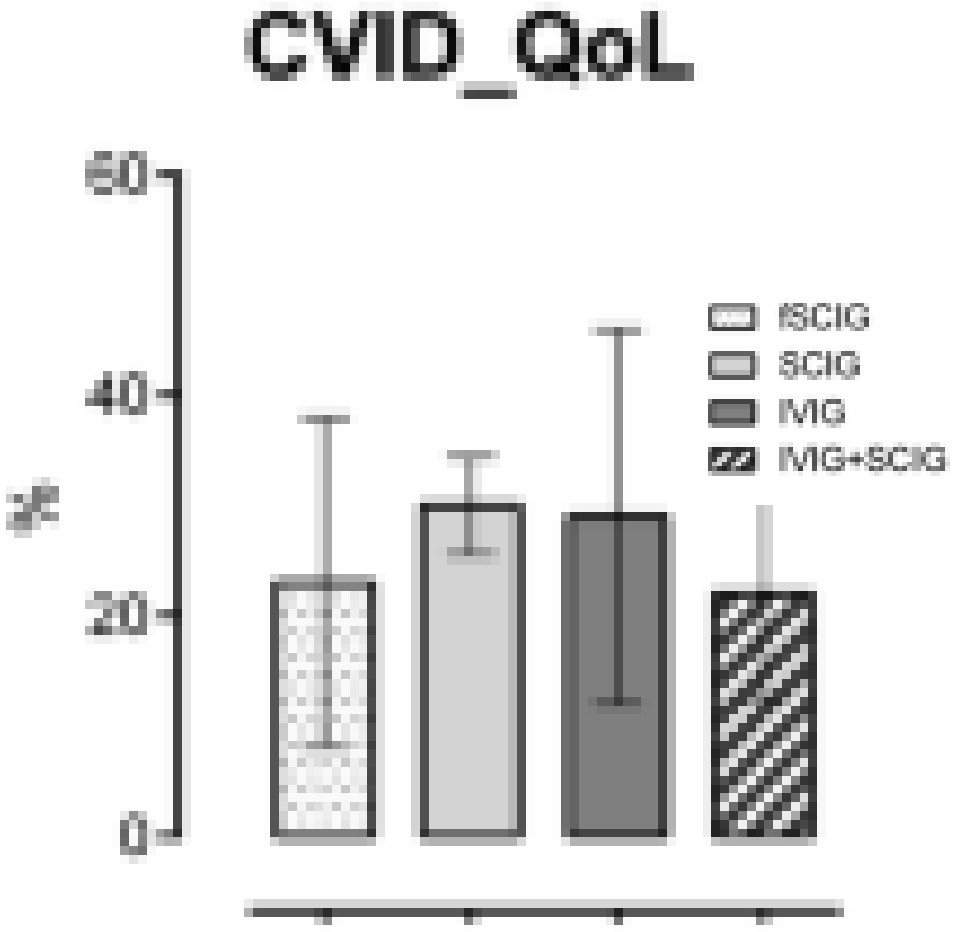


Figure 1 Health of patients with primary immunodeficiency disease by country and route of immunoglobulin administration (visual analog scale).[a]
Notes: [a]EQ-5D questionnaire; [b]EU5 indicates UK, France, Germany, Spain, and Italy.
Abbreviations: Ig, immunoglobulin; IV, intravenous; SC, subcutaneous; EQ-5D, EuroQoL 5 Dimensions.

Patient Preference and Adherence 2014:8 621–629

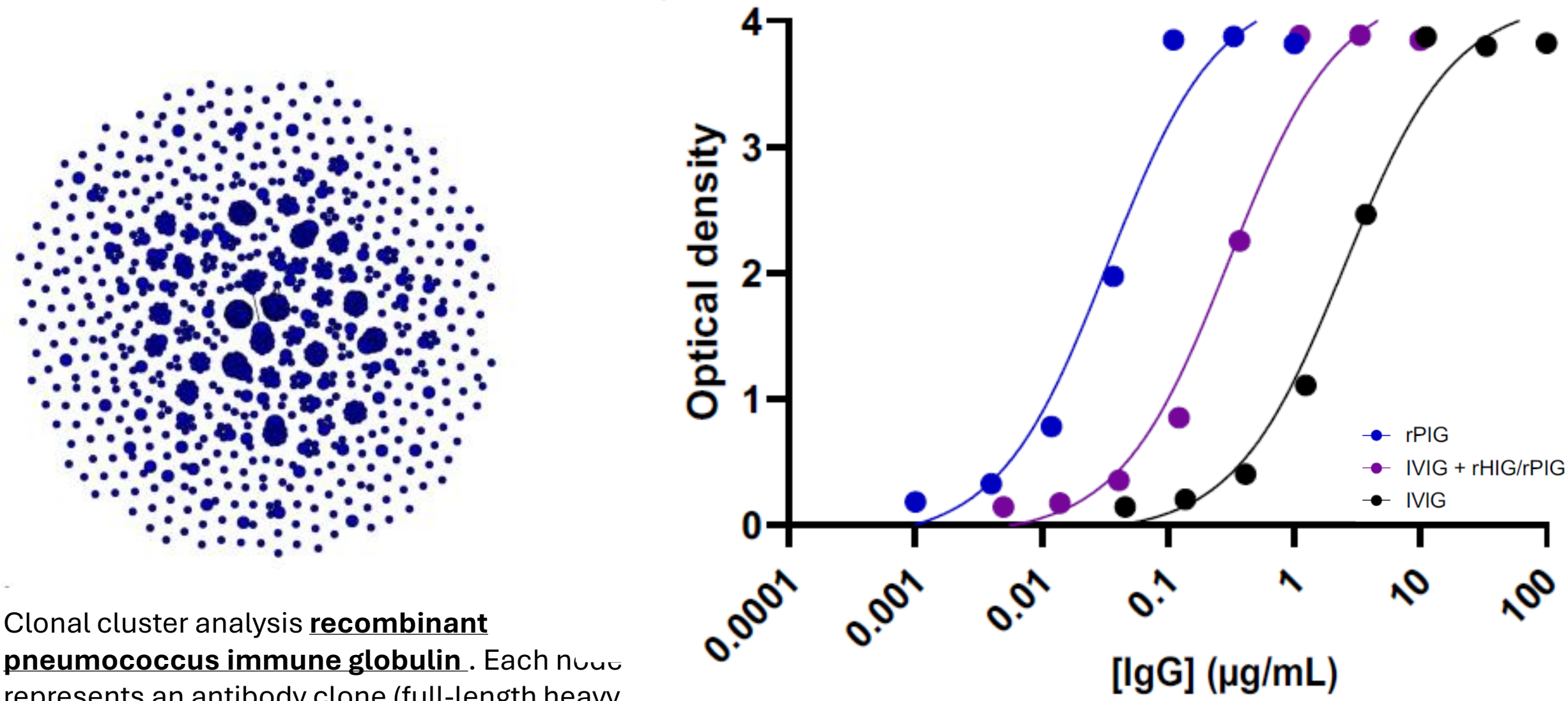
HRQoL Patients with CVID
Different Schedules of Immunoglobulin Administration
Prospective Multicenter Study



mean (SD)	**IVIG concentration**		**P value**
	10 (g/dL)	**5 (g/dL)**	
Age, year	48.0 (15.3)	51.4 (15.2)	0.314
Cumulative monthly Ig dose (mg/kg)	336.8 (167.3)	339.1 (136.8)	0.943
Number of monthly administrations	1.9 (0.7)	2.0 (0.9)	0.894
Patients receiving antibiotic prophylaxis; n (%)	5 (19.2)	0 (0)	0.948
Time (years) from CVID diagnosis	12.5 (11.3)	14.9 (11.1)	0.337
IgG trough serum levels (mg/dl)	670.0 (191.1)	672.4 (148.4)	0.946
All infections. episodes-year	5.3 (5.2)	4.9 (4.8)	0.718
COPD; n (%)	30 (30.0)	16 (61.5)	0.405
CVID-complication, cumulative number	2.3 (1.4)	2.2 (1.6)	0.842
CVID_QOL (%)	**27 (15)**	**29 (18)**	**0.635**

J Clin Immunol 39, 159–170 (2019)

Generation and characterization of a recombinant hyperimmune globulin for PID.



Clonal cluster analysis **recombinant pneumococcus immune globulin** . Each node represents an antibody clone (full-length heavy chain). Similar results with **recombinant *Haemophilus* immune globulin**

Pneumococcal or Hib antibody binding of IVIG + rPIG by ELISA. Binding of serially diluted rPIG, IVIG + rPIG, and IVIG was measured by ELISA

Nat Biotechnol 39, 989–999 (2021). https://doi.org/10.1038/s41587-021-00894-8

Latent therapeutic demand for IG in the USA
N*europathies*

Condition	Mean LTD immunoglobulin g/10^3 population
Chronic inflammatory demyelinating polyneuropathy (CIDP)	83.05 ± 24.5
Guillain–Barré syndrome (GBS)	6.1 ± 3.2
Multifocal motor neuropathy (MMN)	36.1 ± 25.5

NB

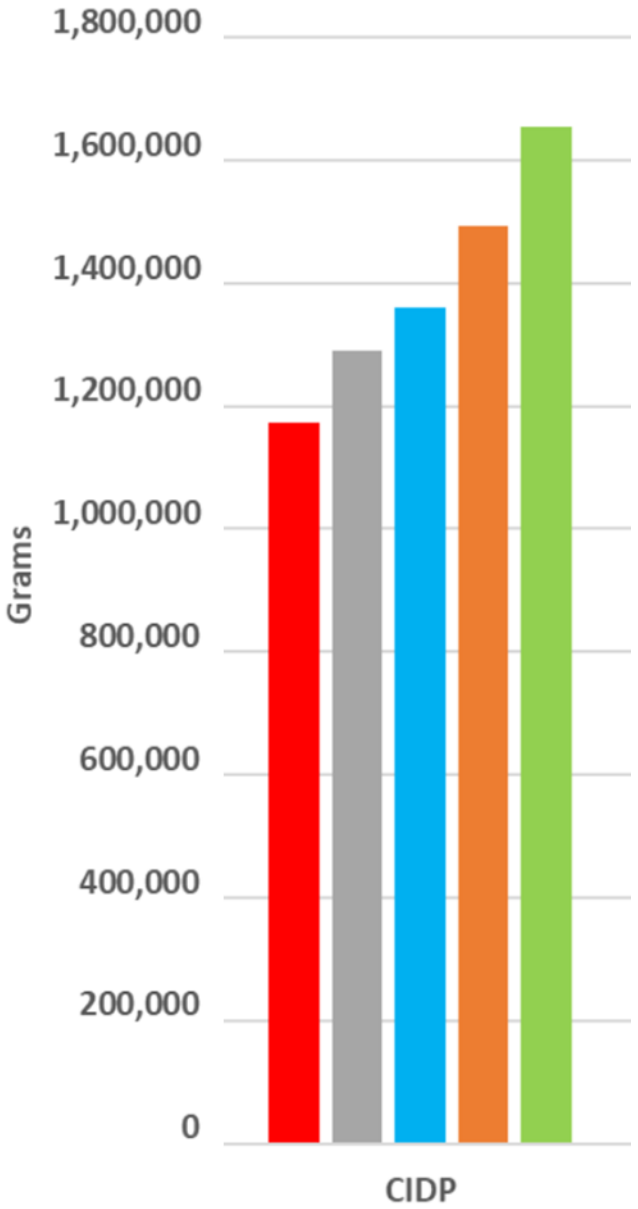
Vox Sang. 2022;117:208–219.

Authors' conclusions

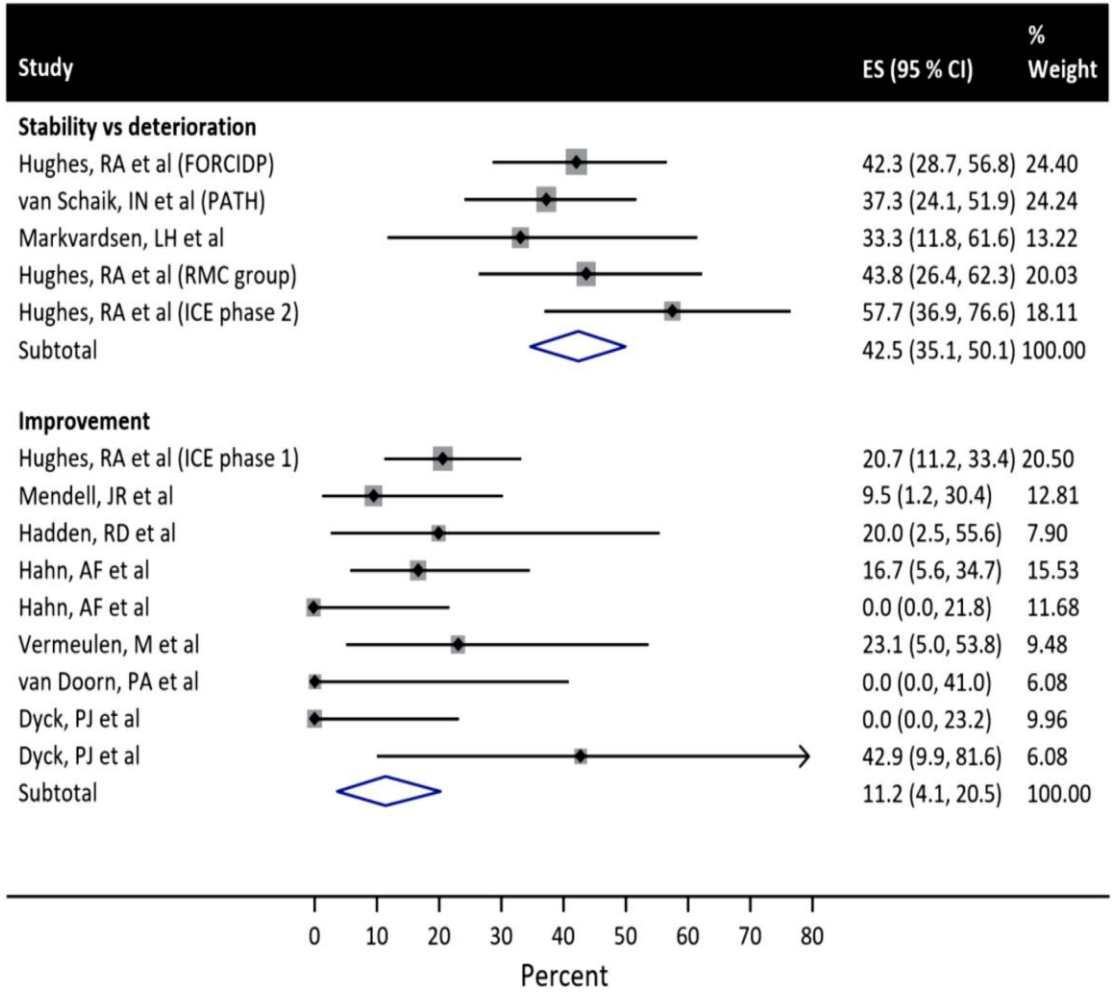
The evidence from RCTs shows that IVIg improves disability for at least two to six weeks compared with placebo, with an NNTB of three***. During this period it has similar efficacy to plasma exchange, oral prednisolone and intravenous methylprednisolone***. In one large trial, the benefit of IVIg persisted for 24 and possibly 48 weeks. Further research is needed to compare the long-term benefits as well as side effects of IVIg with other treatments.

IG usage in CIDP – The Australian case

- Number of patients prescribed Ig 2017-2018 = 2795
- Prevalence estimate using EFNS/PNS criteria = 518-1593

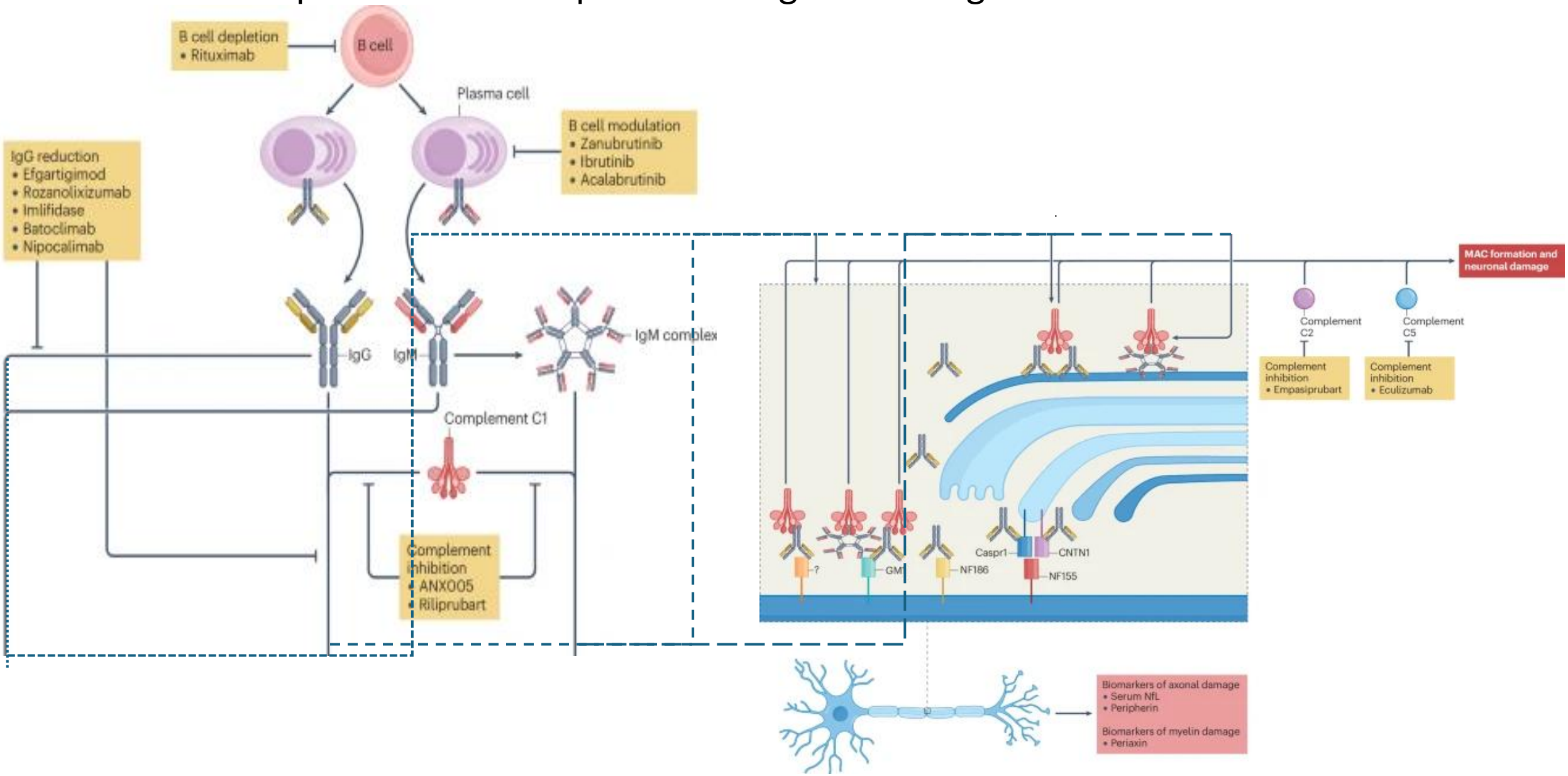


Placebo effect in CIDP



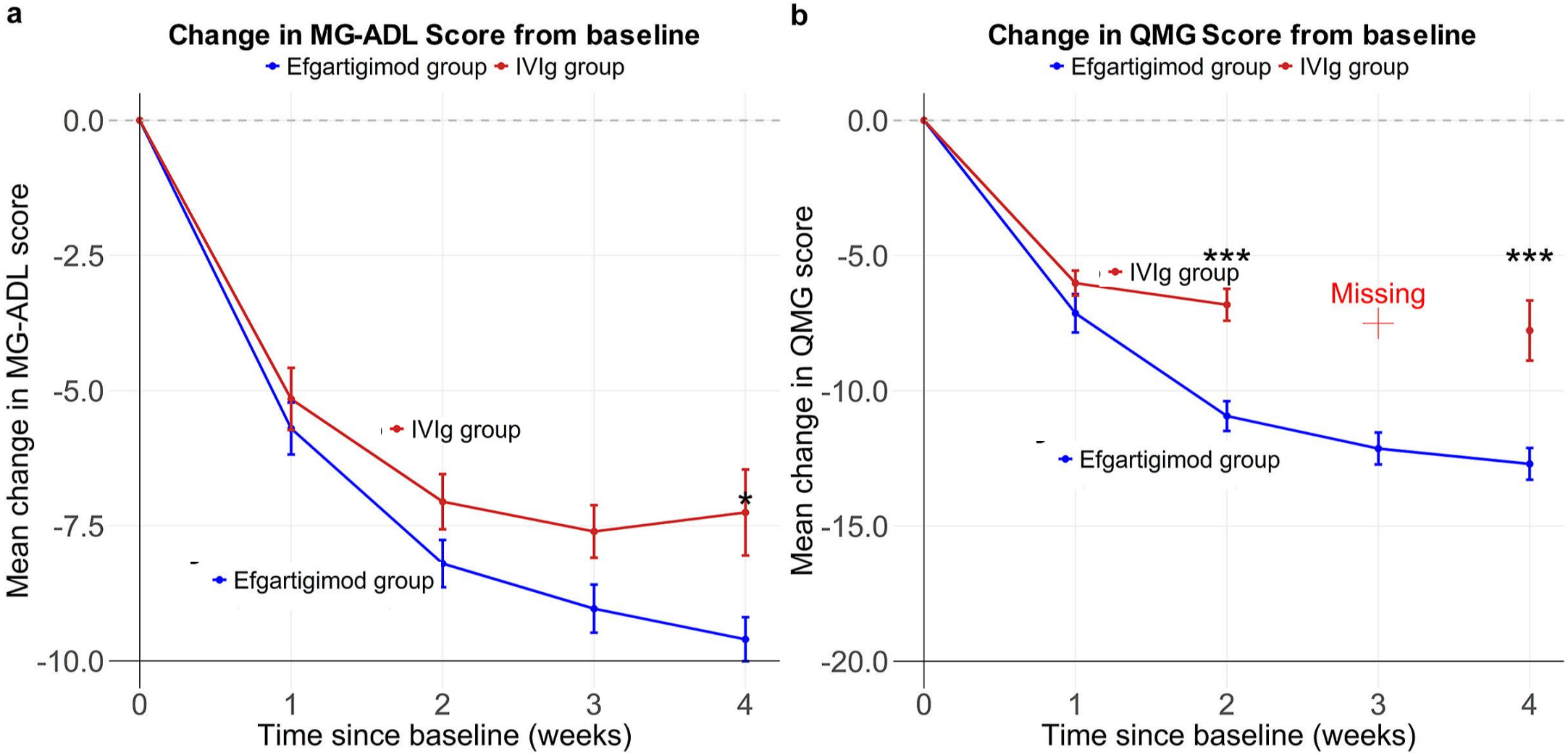
J Peripheral Nervous Sys, Volume: 25, Issue: 3, Pages: 230-237, First published: 05 July 2020, DOI: (10.1111/jns.12402)

Current understanding of pathophysiological mechanisms in primary autoimmune neuropathies and therapeutic strategies that target these mechanisms.



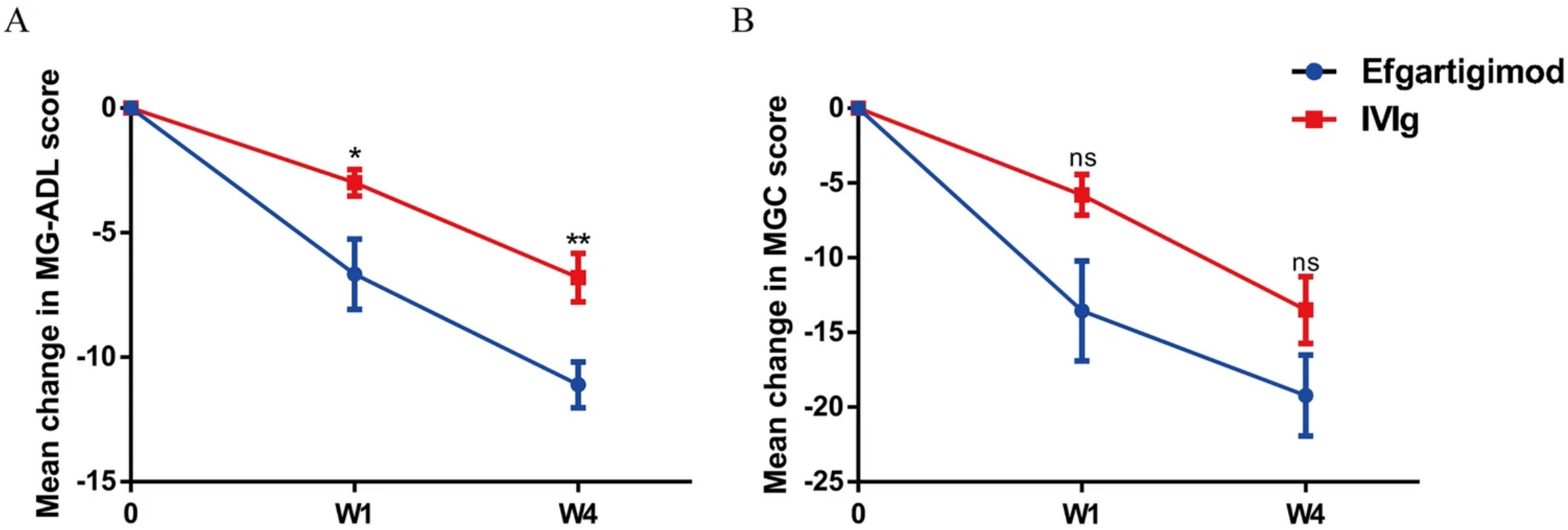
Caballero-Ávila, M., Pascual-Goñi, E., Lleixà, C. et al. The changing landscape of primary autoimmune neuropathies. Nat Rev Neurol 21, 544–555 (2025). https://doi.org/10.1038/s41582-025-01133-3

Efficacy and safety of efgartigimod versus intravenous immunoglobulin in early intervention of acetylcholine receptor antibody-positive impending myasthenic crisis: A *retrospective cohort* study



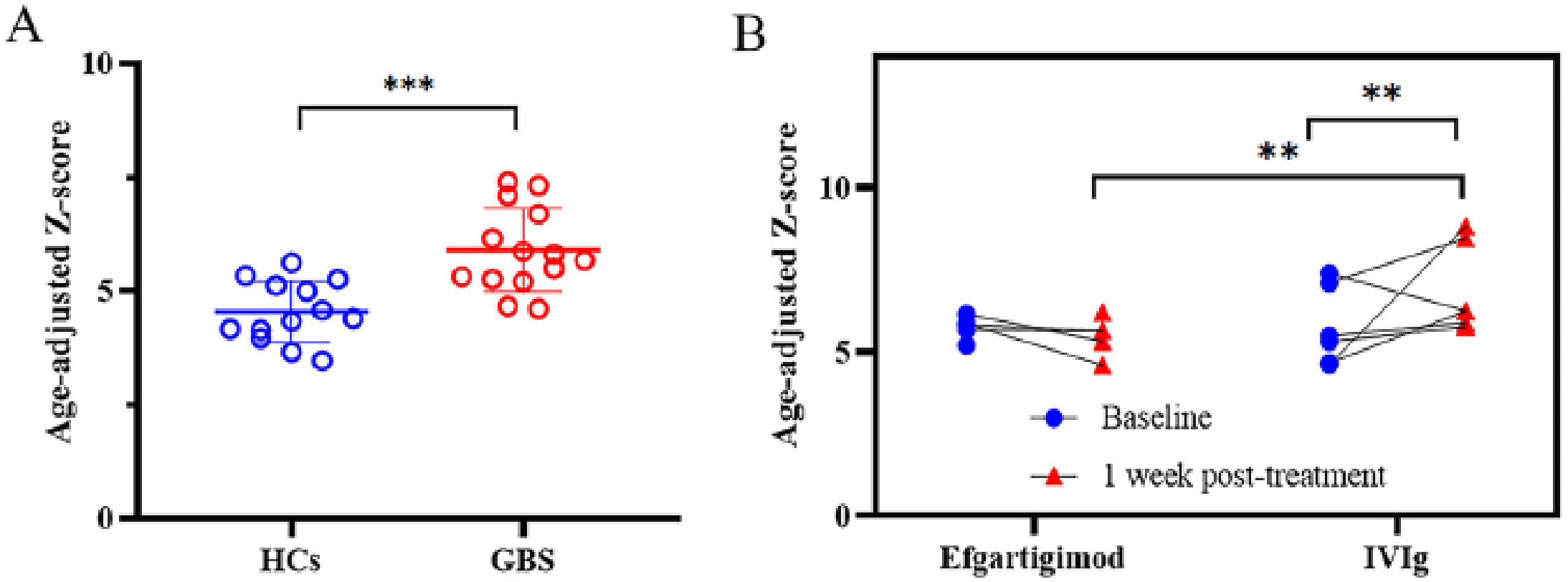
Neurotherapeutics 22 (2025) e00730

Efgartigimod versus IVIG in the treatment of patients with impending myasthenic crisis



Ma, J., Zhang, H., Zhao, J. et al. Efgartigimod versus intravenous immunoglobulin in the treatment of patients with impending myasthenic crisis. Sci Rep 14, 28394 (2024). https://doi.org/10.1038/s41598-024-79918-7

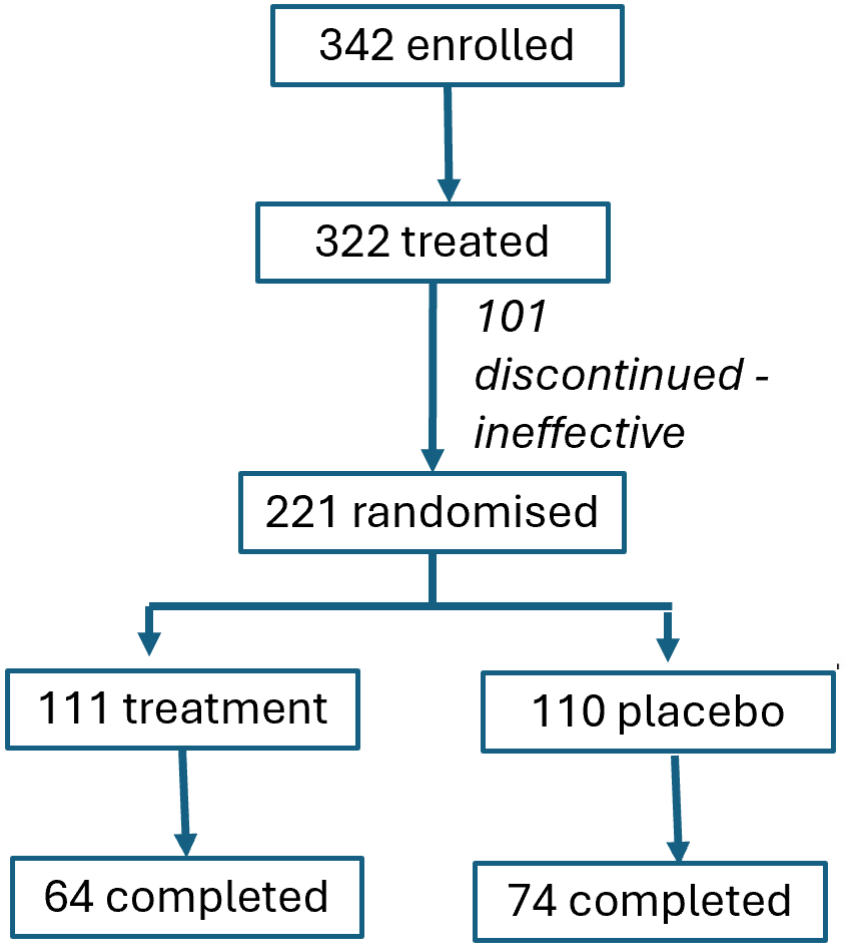
Comparison of intravenous efgartigimod and IVIG in GBS

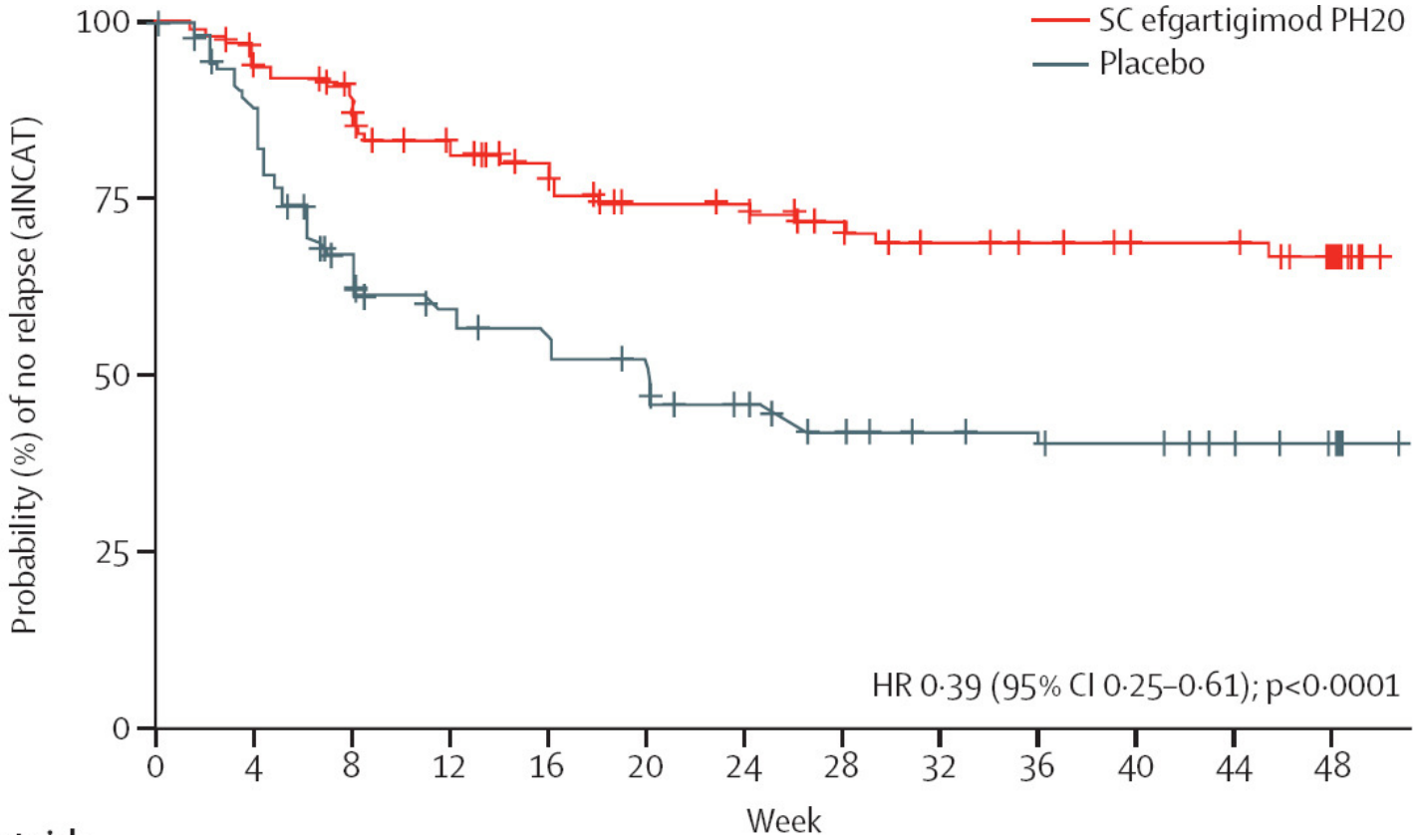


Zhang et al. Orphanet Journal of Rare Diseases (2025) 20:529
https://doi.org/10.1186/s13023-025-04060-0

ADHERE trial for S.C. Efgartigomod (Vyvgart) in CIDP
Relative risk of relapse based on time to first aINCAT deterioration

Trial design (simplified)





"ADHERE showed the efficacy of subcutaneous efgartigimod PH20 in reducing the risk of relapse versus placebo in people with CIDP who responded to treatment. Further studies are needed to provide data on the longer-term effects of efgartigimod alfa and how it compares with currently available treatment options."

The Lancet Neurology, Volume 23, Issue 10, 1013 - 1024

Regulatory status

U.S. FOOD & DRUG ADMINISTRATION

Home / Drugs / News & Events for Human Drugs / FDA approves treatment for chronic inflammatory demyelinating polyneuropathy (CIDP) in adults

FDA approves treatment for chronic inflammatory demyelinating polyneuropathy (CIDP) in adults

News & Events for Human Drugs

Meetings, Conferences, & Workshops

Q&A with FDA Podcast

CDER Conversations

From Our Perspective

Action

The U.S. Food and Drug Administration has approved Vyvgart Hytrulo (efgartigimod alfa and hyaluronidase-qvfc) for the treatment of chronic inflammatory demyelinating polyneuropathy (CIDP) in adults. Vyvgart Hytrulo is a combination of efgartigimod alfa, a neonatal Fc receptor blocker, and hyaluronidase, an endoglycosidase. Vyvgart Hytrulo was previously approved for the treatment of adults with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive.

For CIDP, Vyvgart Hytrulo is administered subcutaneously (under the skin) as weekly injections. The recommended dose is detailed in the prescribing information.

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Vyvgart

efgartigimod alfa

Medicine | Human

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Authorised
This medicine is authorised for use in the European Union

Application under evaluation — CHMP opinion — **European Commission decision**

Page contents

- Overview
- Product information
- Product details
- Authorisation details
- Assessment history

Overview

Vyvgart is a medicine for treating adults with:

- generalised myasthenia gravis (a disease that leads to muscle weakness and tiredness) and whose immune system (the body's defence system) produce antibodies against a protein called acetylcholine receptor, found on muscle cells. It is given together with other medicines used for the treatment of myasthenia gravis;
- chronic inflammatory demyelinating polyneuropathy (CIDP), a disease in which the immune system works abnormally and destroys the protective covering around the nerves. It is used in patients whose disease is worsening or came back and who have had previous treatment with corticosteroids or immunoglobulins (other medicines used to treat CIDP).

Myasthenia gravis and CIDP are rare, and Vyvgart was designated an 'orphan medicine' for these diseases on 21 March 2018 and 14 January 2022, respectively.

Vyvgart contains the active substance efgartigimod alfa

Moving beyond immunoglobulin therapy for CIDP with efgartigimod

*Lünemann, J.D. Moving beyond immunoglobulin therapy for CIDP with efgartigimod. Nat Rev Neurol **21**, 1–2 (2025).*

The design of the ADHERE trial, however, means that, despite the beneficial effects observed, translation into clinical practice will not be straightforward. *The trial design had in-built pre-selection of individuals with active disease and a documented response to efgartigimod before the randomized controlled stage. This enrichment of participants with those whose disease responded to treatment ensured a streamlined design that was valuable given that responses to immunotherapy in CIDP are remarkably heterogeneous, and the approach was compatible with contemporary regulatory and expert consensus recommendations. In this context, however,* ***the general approval of efgartigimod for treatment of CIDP given by the FDA means that a proportion of people with CIDP who receive efgartigimod in daily clinical practice are unlikely to experience meaningful benefits from the treatment****. In the ADHERE trial, the onset of response was generally fast (25% responded within 2 weeks and 50% responded within 4 weeks), so* ***efficient real-word use of efgartigimod will require rigorous clinical monitoring at the initiation of treatment as well as management of patients' expectations and development of a consensus on valid criteria to define a therapeutic response and criteria for discontinuation****.*

Early deterioration of CIDP following transition from IVIG to FcRn inhibitor treatment (FIT)

Clinical features including disability scores before and after FIT treatment and after rescue treatment

Pt	Age/ Sex	Disease Duration (months)	Disease Characteristic	Duration on IVIg (months)	INCAT at onset	INCAT on IVIg	INCAT after FIT	Rescue Treatment	Time on Rescue therapy	INCAT after rescue
1	68/F	36	Classic	12	5	2	7	Plex, IVIg, Pred	9 wks	3
2	74/M	84	Distal	84	4	1	6	IVIg	8 wks	3
3	58/F	180	Classic	120	8	2	4	Plex,IVIg, Pred	6 wks	5
4	47/F	60	Multifocal	48	5	2	6	IVIg, Pred	5 wks	4

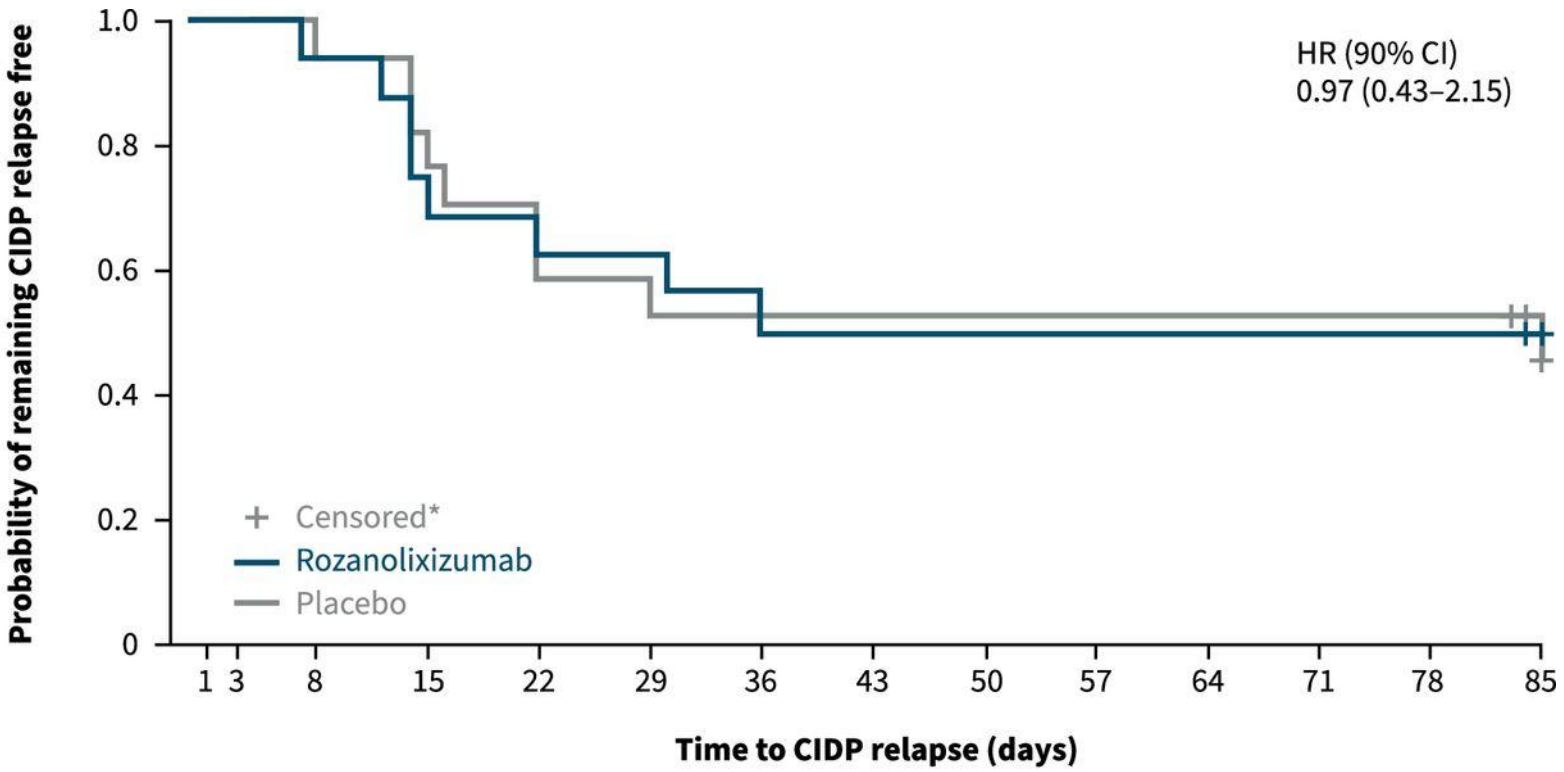
".....the transition from IVIG to efgartigimod in a real world setting was not studied in the pivotal trial. We have treated nine patients with FIT in our practice and report findings of four of those patients who had severe relapse of CIDP after treatment. Five of the other patients neither improved nor declined with FIT. This raises questions about the issues related to transitioning patients from IVIG to efgartigimod..... the transition of patients with CIDP who are stable on IVIG therapy to FIT can lead to severe worsening of disease "

Journal of the Neurological Sciences 468 (2025) 123313

The report,,,, highlights critical safety concerns about transitioning patients from immunoglobulin therapy to efgartigimod..... A post-hoc analysis examining recognizable subsets.....is needed to improve diagnostic precision and strengthen confidence in efgartigimod's role in CIDP. Addressing these issues will only ensure that both patients and clinicians benefit from a clearer, more accurate understanding of promising therapies.

Journal of the Neurological Sciences 468 (2025) 123355

Randomised double blind Phase 2a trial of rozanolixizumab in patients with CIDP



FDA and EMA approval for MG BUT seems ineffective for CIDP

Luis Querol et al. J Neurol Neurosurg Psychiatry 2024;95:845-854

Johnson&Johnson

imaavy™ (nipocalimab-aahu)

Johnson & Johnson receives FDA approval for IMAAVY™ (nipocalimab-aahu), a new FcRn blocker offering long-lasting disease control in the broadest population of people living with generalized myasthenia gravis (gMG)

ClinicalTrials.gov

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Home > Search Results > Study Record

The U.S. government does not review or approve the safety and science of all studies listed on this website.

Read our full disclaimer for details.

Recruiting

Efficacy and Safety Study of Nipocalimab for Adults With Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

ClinicalTrials.gov ID NCT05327114

Sponsor Janssen Research & Development, LLC

Information provided by Janssen Research & Development, LLC (Responsible Party)

Last Update Posted 2025-11-07

IMMUNOVANT

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IMMUNOVANT ANNOUNCES POSITIVE RESULTS FOR BATOCLIMAB MYASTHENIA GRAVIS (MG) AND CHRONIC INFLAMMATORY DEMYELINATING POLYNEUROPATHY (CIDP) STUDIES

March 19, 2025 7:45 am EDT

Download as PDF

- Pivotal study in MG met primary endpoint of change from baseline in MG-ADL in ChIR population at 12 weeks, with a 5.6 point improvement in the higher dose arm (with 74% mean IgG reduction) and a 4.7 point improvement in the lower dose arm (with 64% mean IgG reduction)
- Initial CIDP results from Period 1, following standard of care washout, demonstrate a mean improvement in the adjusted INCAT disability score of 1.8 across batoclimab arms and an 84% responder rate in those patients who achieved an IgG lowering greater than 70%

Read our full disclaimer for details.

Active, not recruiting

To Assess Efficacy and Safety of Batoclimab in Adult Participants With Active CIDP

ClinicalTrials.gov ID NCT05581199

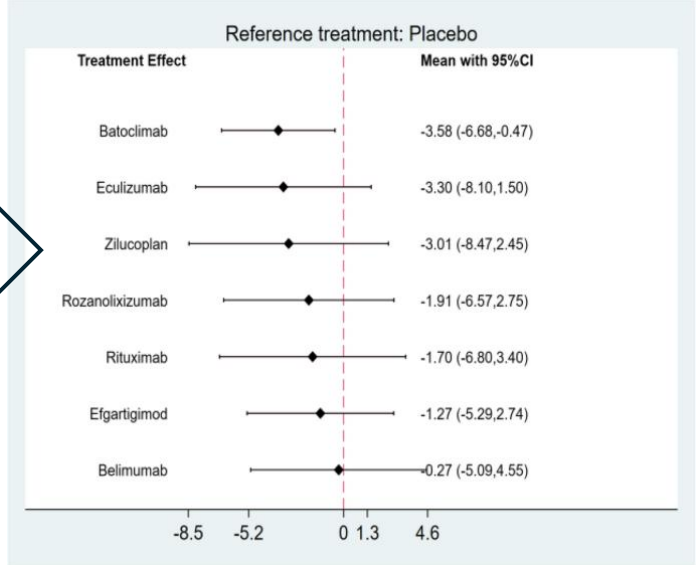
Sponsor Immunovant Sciences GmbH

Information provided by Immunovant Sciences GmbH (Responsible Party)

Last Update Posted 2024-11-25

"At present, Immunovant does not intend to seek regulatory approval for batoclimab in MG or CIDP..."
WHY NOT?

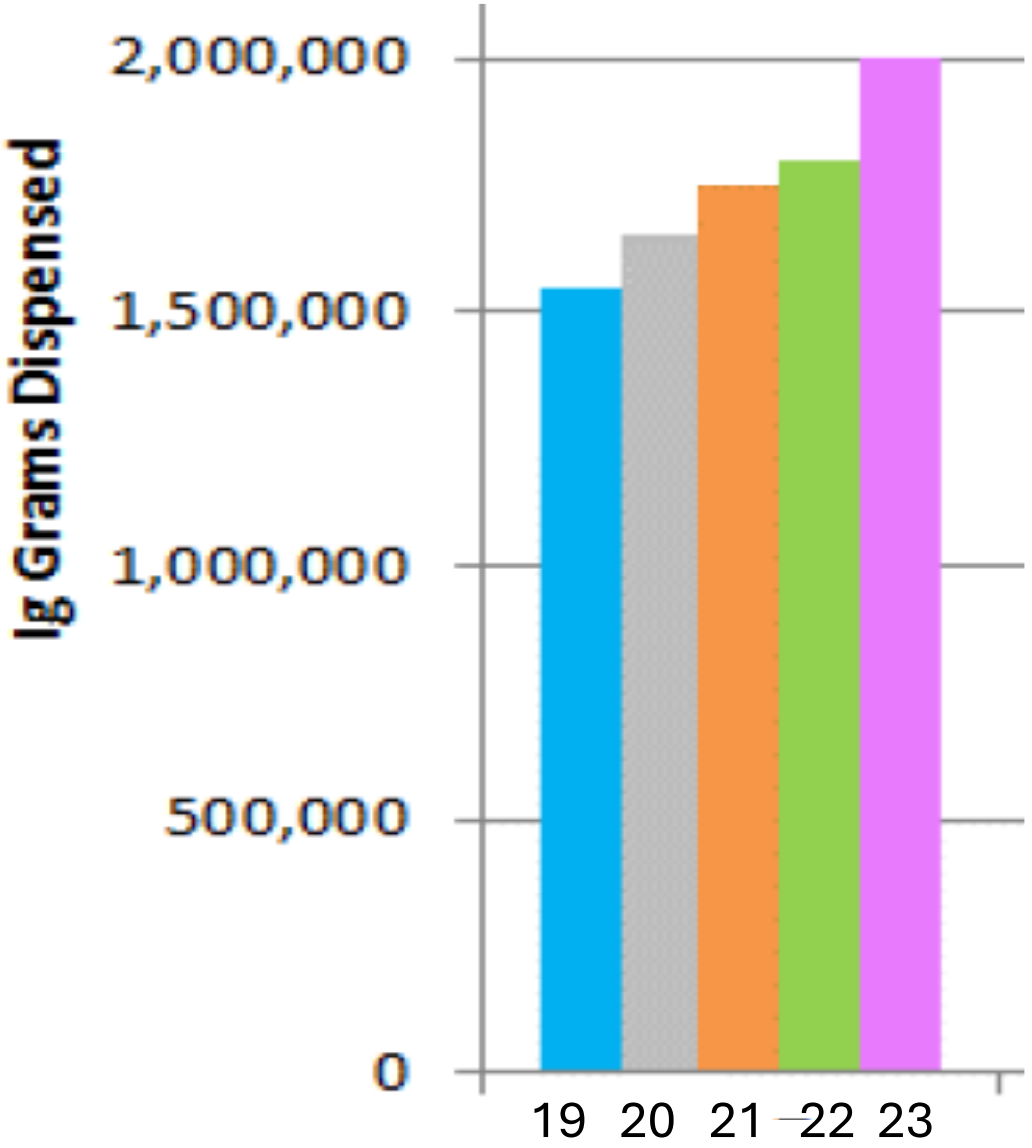
RANKING IN MGC SCORE



Syst Rev **14**, 106 (2025). https://doi.org/10.1186/s13643-025-02859-3

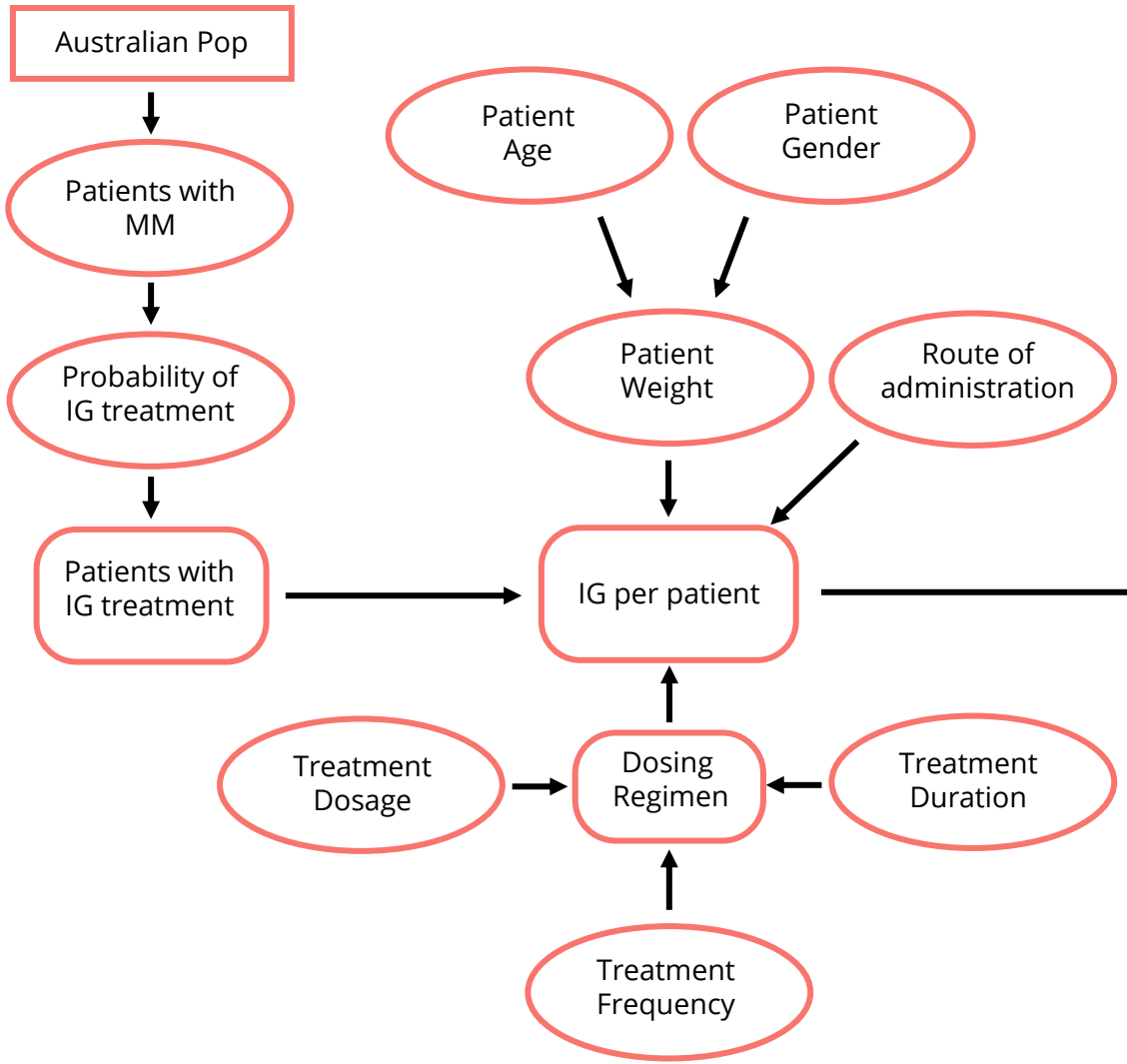
IG issues – Australia - Acquired hypogammaglobulinaemia —

“haematological malignancy and post haemopoietic stem cell transplantation (HSCT)”

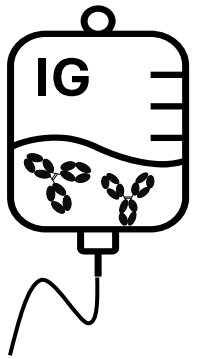


THE HIGHEST USING GROUP OF INDICATIONS IN AUSTRALIA

Modelling the LTD for IG in Multiple Myeloma in Australia



Latent Therapeutic Demand



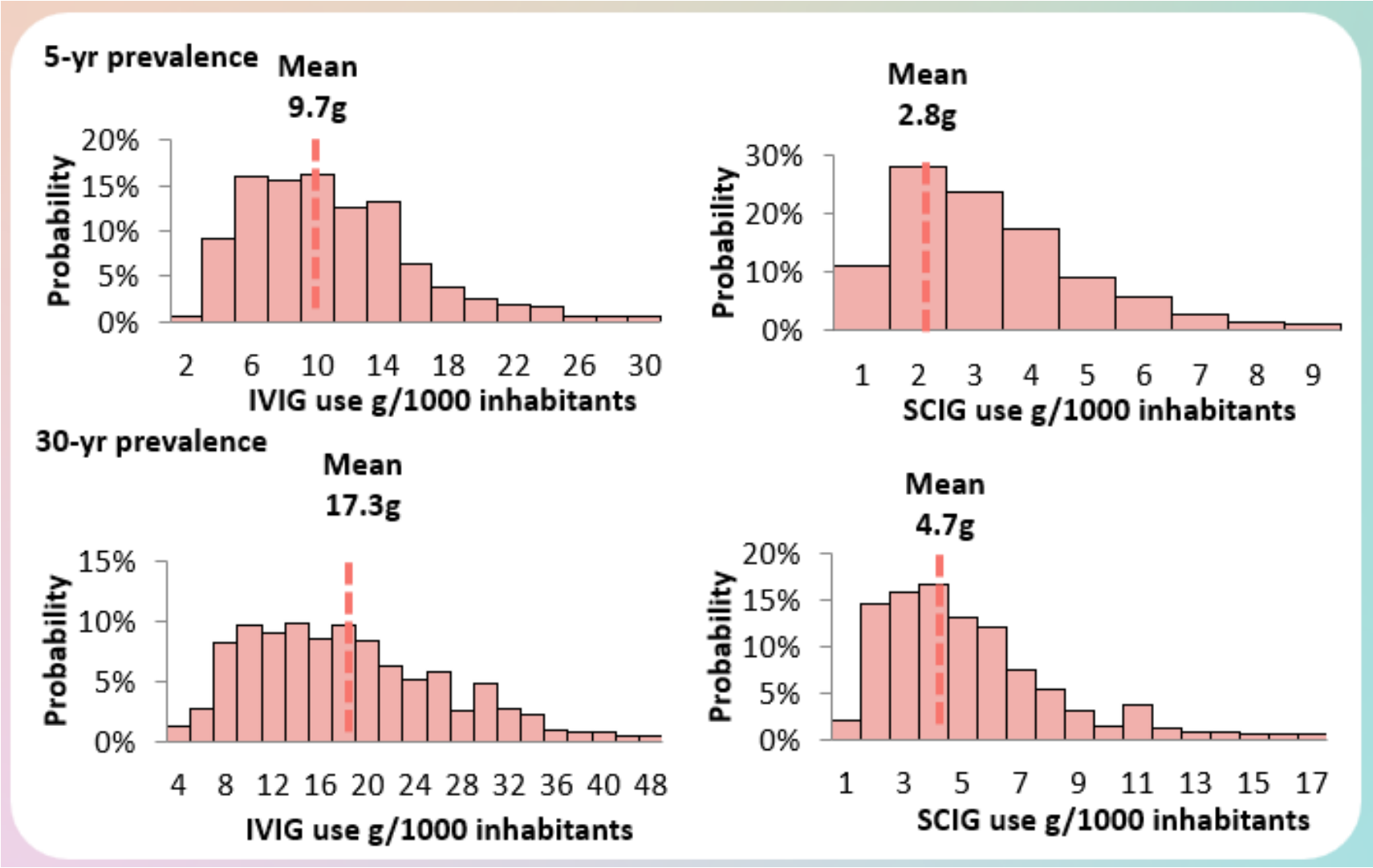
Base case estimate

1. **5yr prevalence**
17g / 1000 inhabitants

2. **30yr prevalence**
28g / 1000 inhabitants

Actual usage (2023-4)
23g / 1000 inhabitants

Current usage (23g) is comparable to what is estimated for a 30-yr prevalence LTD (22 g)

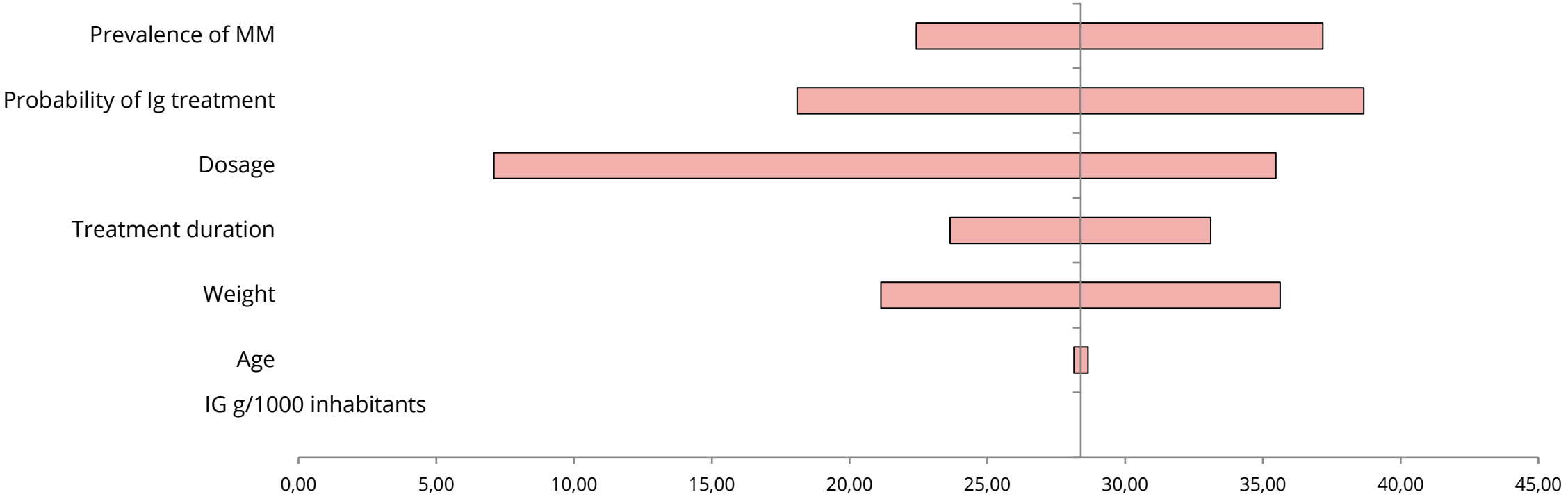


Total IG Usage

12.5 g/10^3

22 g/10^3

Modelling the LTD for IG in Multiple Myeloma in Australia
One-way sensitivity analysis



NATIONAL BLOOD AUTHORITY
AUSTRALIA

Jo Das
Senior Research Advisor
The University of Western Australia
Office of Research
Perth WA 6009

Dear Jo Das

NATIONAL BLOOD SECTOR RESEARCH AND DEVELOPMENT GRANT

Thank you for the application in response to our request for submissions for a National Blood Sector Research and Development Grant.

After careful evaluation of all responses received, I am pleased to inform you that the submission by Prof Albert Farrugia from the University of Western Australia for a Project Grant titled **'Estimation and application of the latent therapeutic demand for immunoglobulin therapy in acquired and secondary immunodeficiencies in Australia'** has been successful.

Please provide your formal acceptance of this offer by email. If we have not received your acceptance by 5 February 2026, this offer will expire.

We will commence work with you to complete a Grant Funding Agreement once we receive your response.

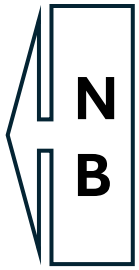
Please note that this offer is being provided under embargo. Grants will only be awarded following successful negotiation and approval of funding agreement by all parties. Details of the Grant Funding Agreement will not be published on the National Blood Authority website until after the agreement has been executed. Therefore, you are required to maintain confidentiality until such time as the embargo has been lifted.

If you have any queries, please contact the Research and Development Officer via

Accessibility: Investigate

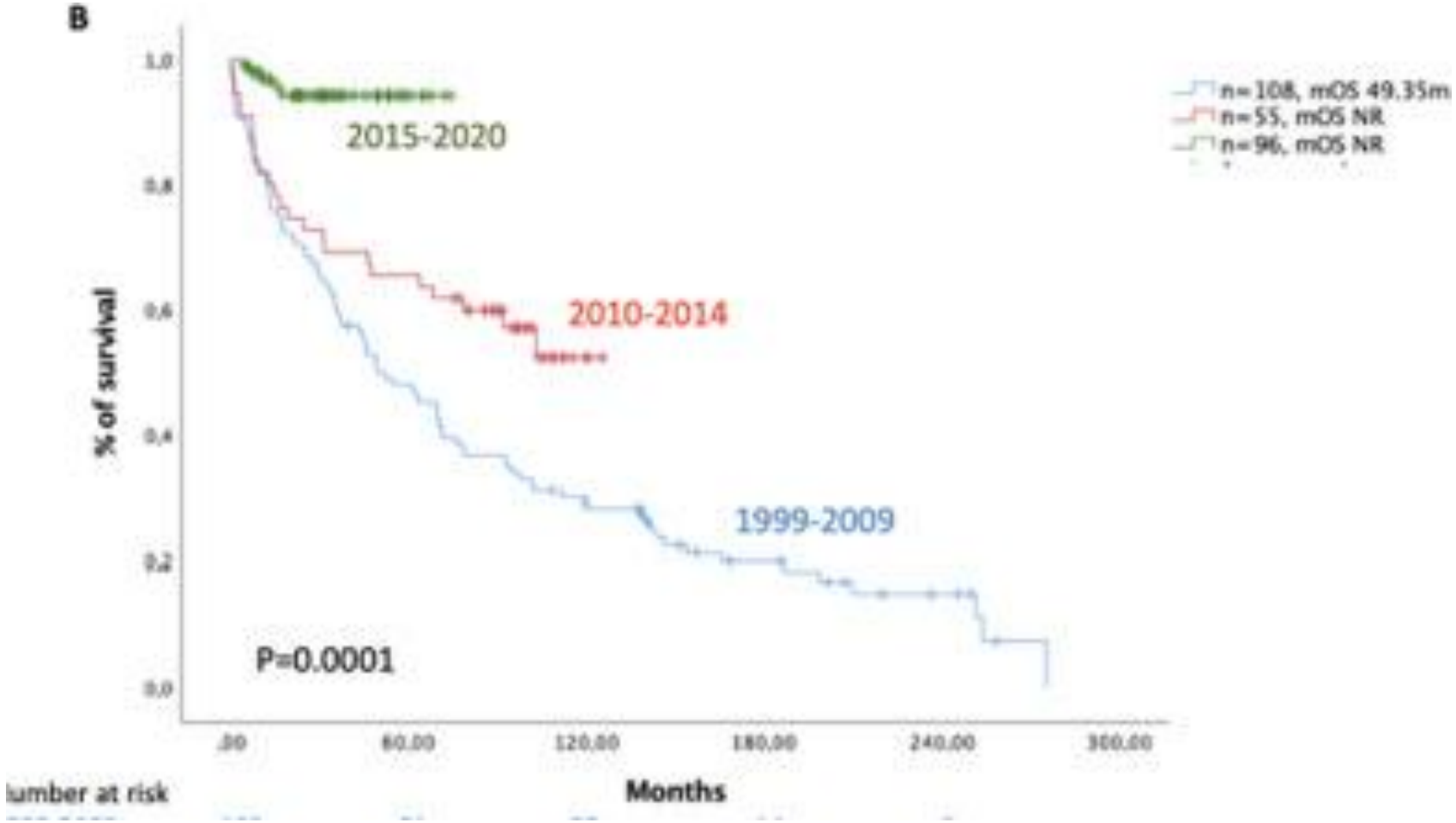
Variation in immunoglobulin use and impact on survival in myeloma

Parameter	Hazard Ratio	95% CI	P
Baseline hypogammaglobulinaemia (<7.0 g/L)	1.02	0.83 – 1.25	
Baseline severe hypogammaglobulinaemia (<4.0 g/L)	1.03	0.82 – 1.3	
Use of immunoglobulin	0.73	0.46 – 1.16	0.16
Use of immunoglobulin with baseline hypogammaglobulinaemia	0.80	0.39 – 1.63	0.54
Use of immunoglobulin with severe baseline hypogammaglobulinaemia	0.91	0.40 – 2.10	0.83
Use of immunoglobulin – infection-related death	1.30	0.50 – 3.30	
Use of immunoglobulin – progression-free survival	0.85	0.57 – 1.28	



eJHaem. 2024;5:690–697

Survival in multiple myeloma
Patients <65 years old



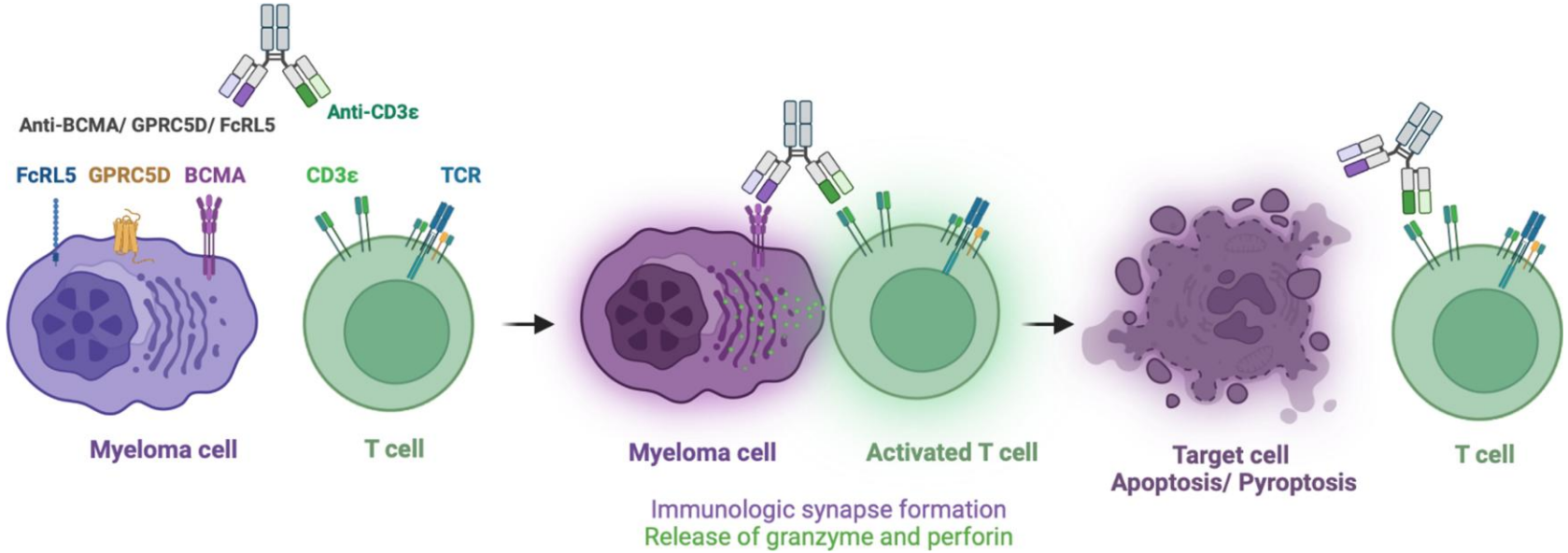
Journal of Hematology & Oncology 2023 16:76

Rapidly emerging therapies for multiple myeloma (and other cancers)

1. Biphasic Mc Abs

Hematology Am Soc Hematol Educ Program 2023; 2023 (1): 332–339. doi: https://doi.org/10.1182/hematology.202300043

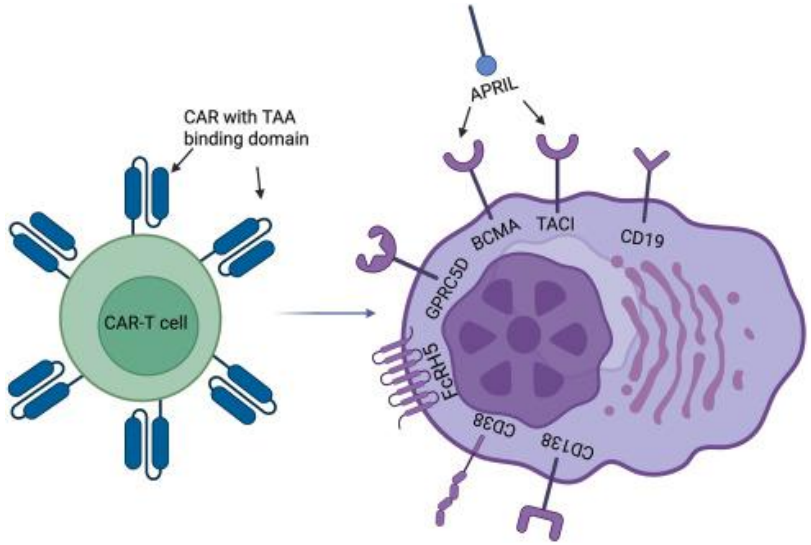
- Teclistamab (TECVAYLI), approved in October 2022
- Elranatamab (ELREXFIO), approved in August 2023
- Talquetamab (TALVEY), approved in August 2023
- Linvoseltamab (LYNOZYFIC), approved in July 2025.



2. CAR T cell therapies

Best Practice & Research Clinical Haematology, Volume 38, Issue 4, 2025, https://doi.org/10.1016/j.beha.2025.101659

Idecabtagene vicleucel and Ciltacabtagene autoleucel approved by FDA



Multiple Myeloma Meeting, Melbourne 21/11/2025

Anti-infective prophylaxis

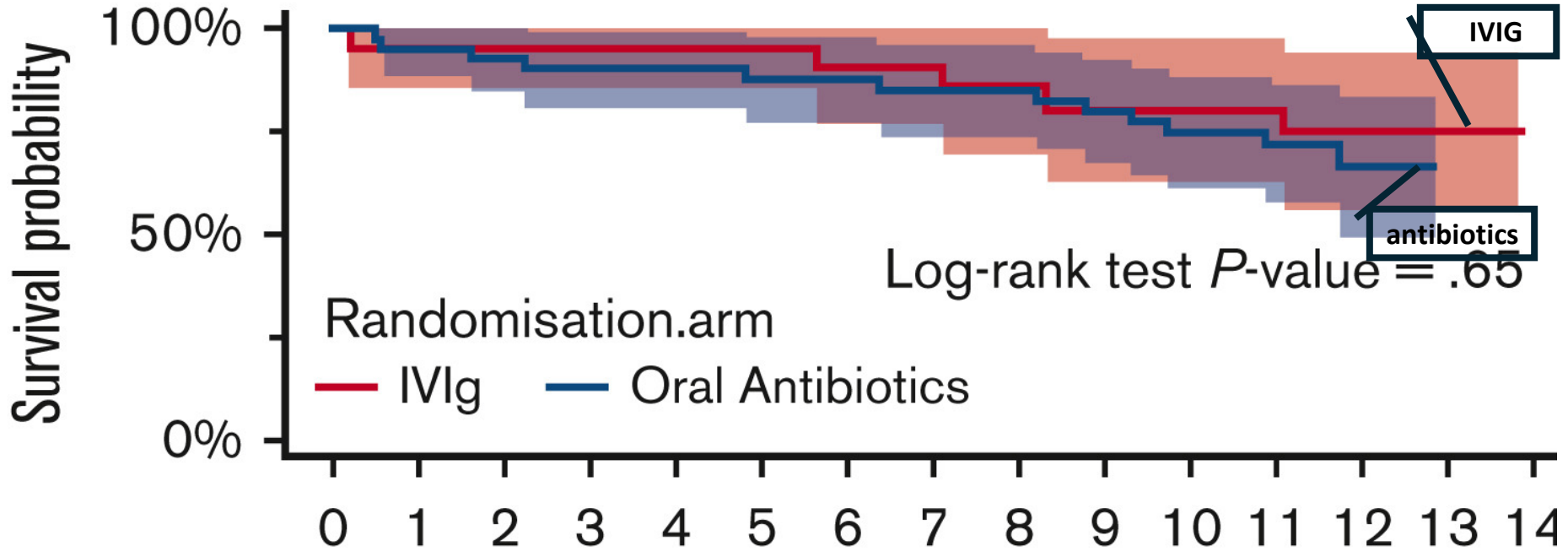
Viral prophylaxis:
Valaciclovir: HSV: 500 mg orally daily; VZV: 500 mg orally TWICE daily
Aciclovir: HSV: 400 mg to 800 mg orally TWICE daily; VZV: 800 mg orally TWICE daily
-Our institution, HSV+VZV covered

PJP Prophylaxis:
1st line: co-trimoxazole
2nd line: dapsone
3rd line: pentamidine or atovaquone
Use will depend on treatment

Fungal prophylaxis:
Fluconazole 400mg daily for neutropenic period
Melphalan ASCT, DT-PACE or DCEP

IG ?

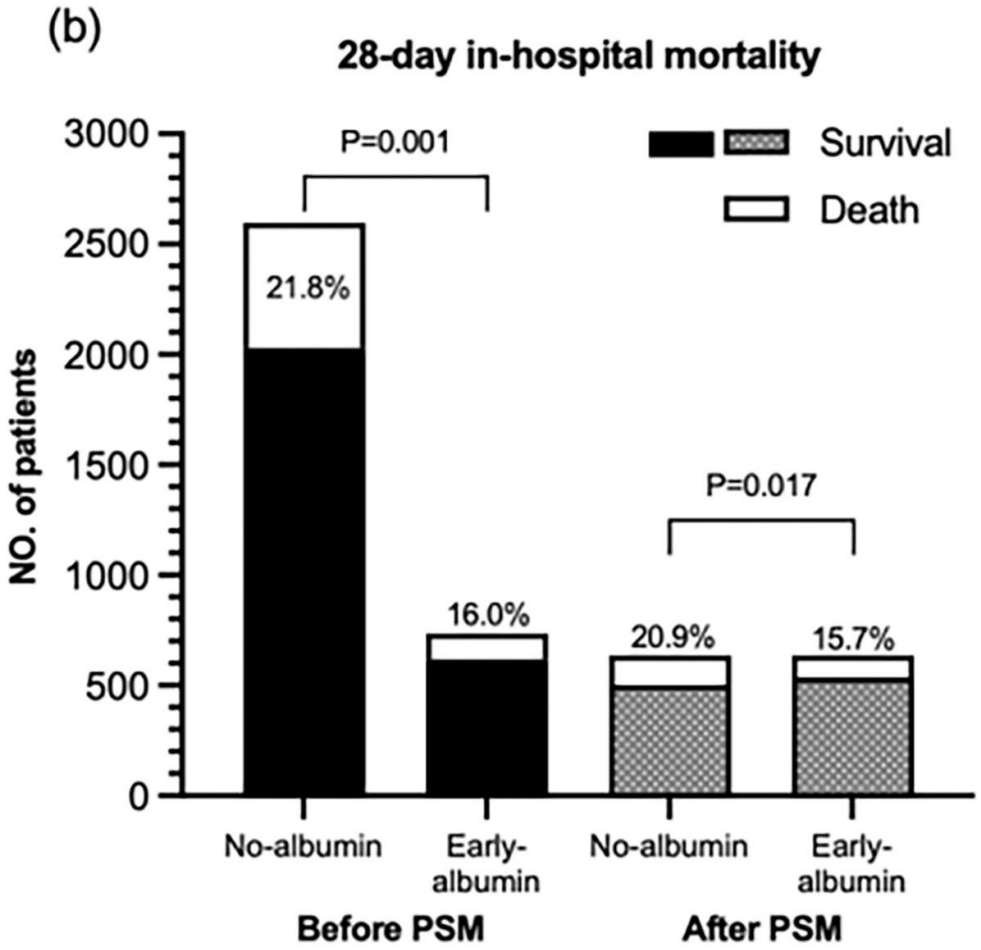
Immunoglobulin replacement vs prophylactic antibiotics for hypogammaglobulinemia secondary to hematological malignancy



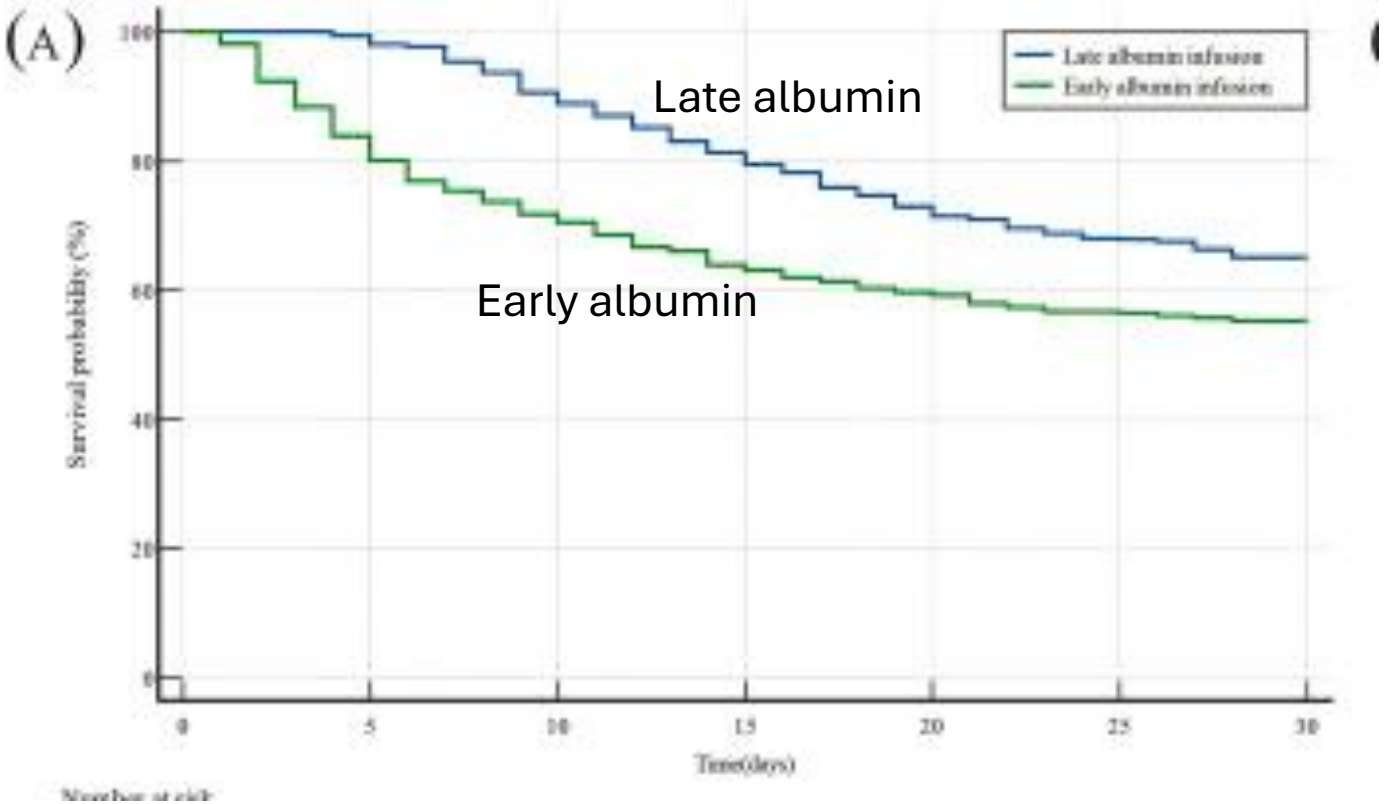
Time to first major infection based on the treatment arm

Blood Advances (8) 9 April 2024, Pages 1787-1795

Albumin in sepsis – Still in equipoise

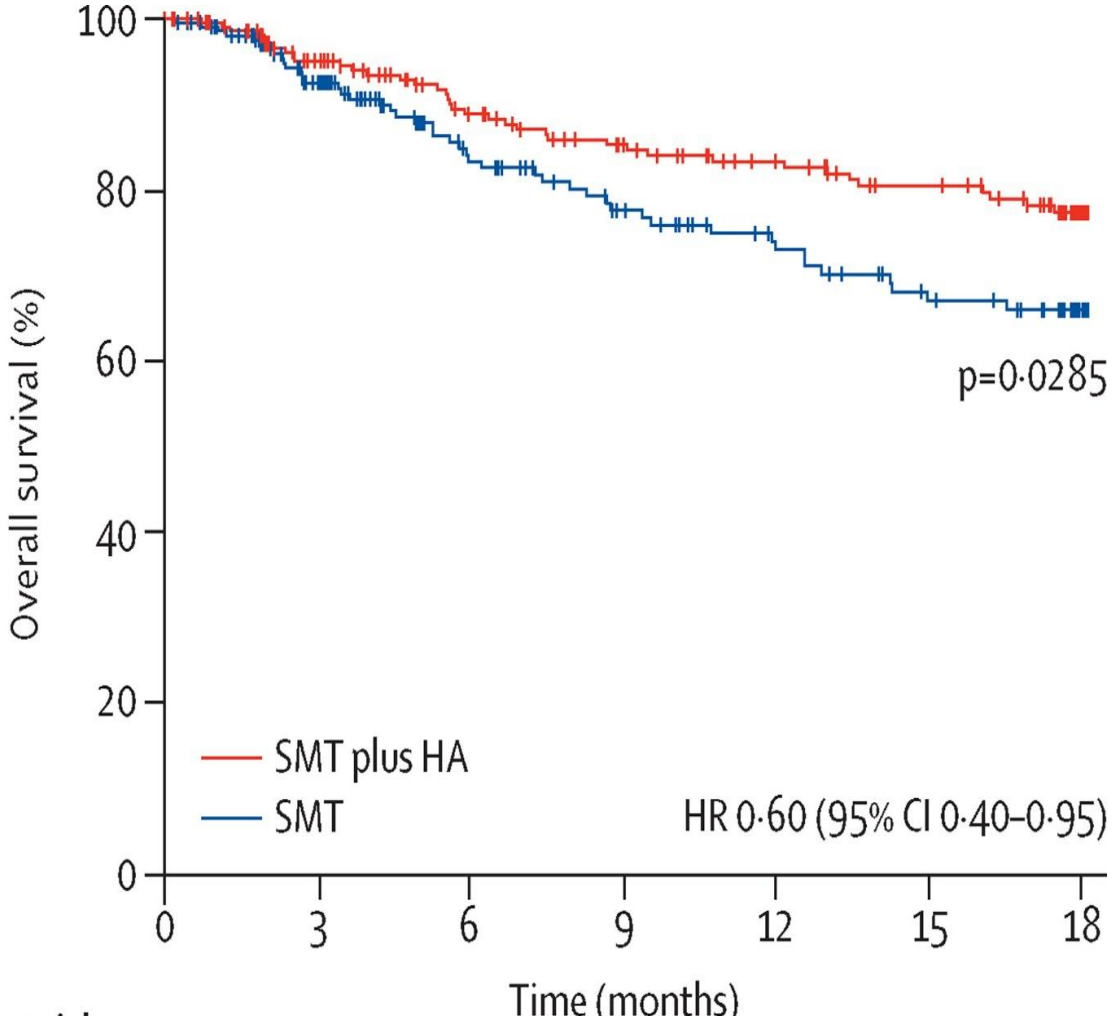


Journal of Intensive Care Medicine. 2025;0(0).

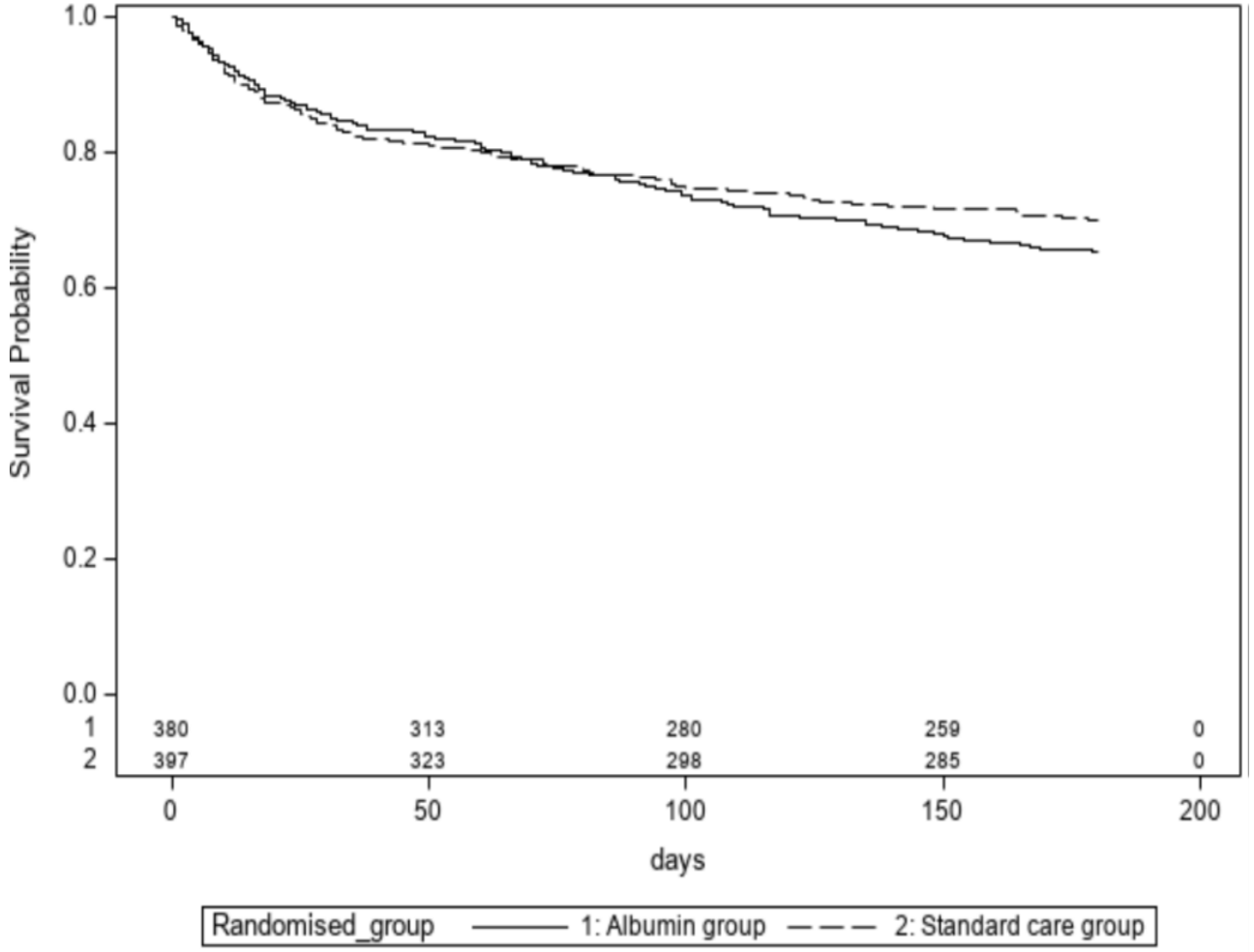


Respiratory Medicine 247 (2025) 108273

Albumin in decompensated cirrhosis – Conflicting studies

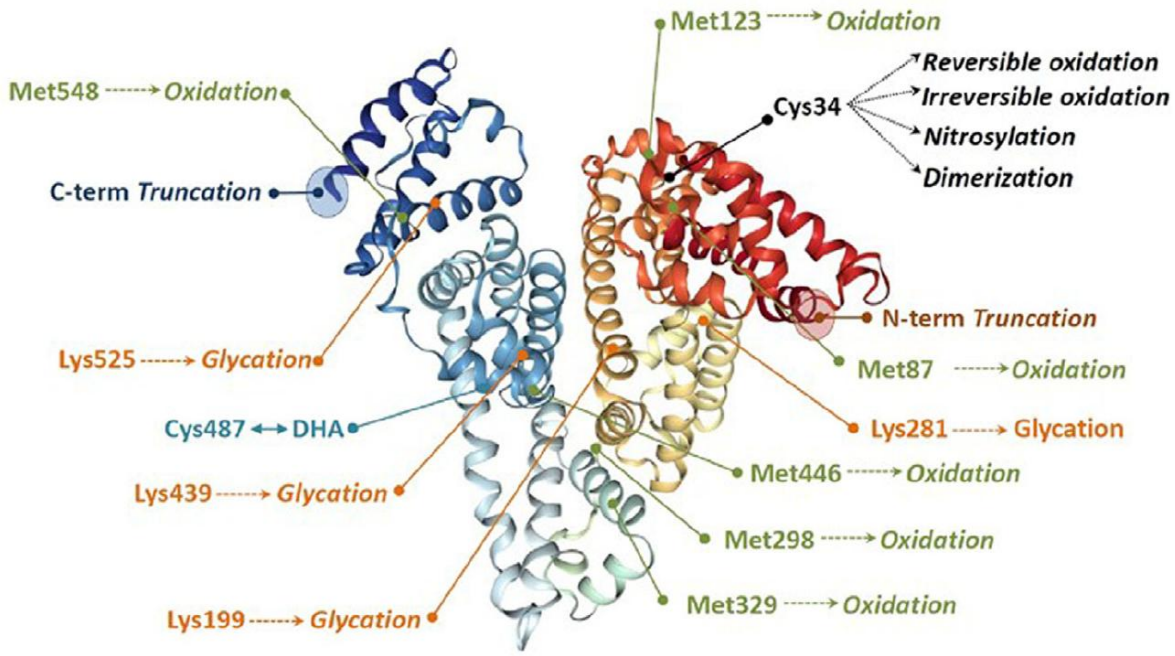


The Lancet, Volume 391, Issue 10138, 2417 - 2429

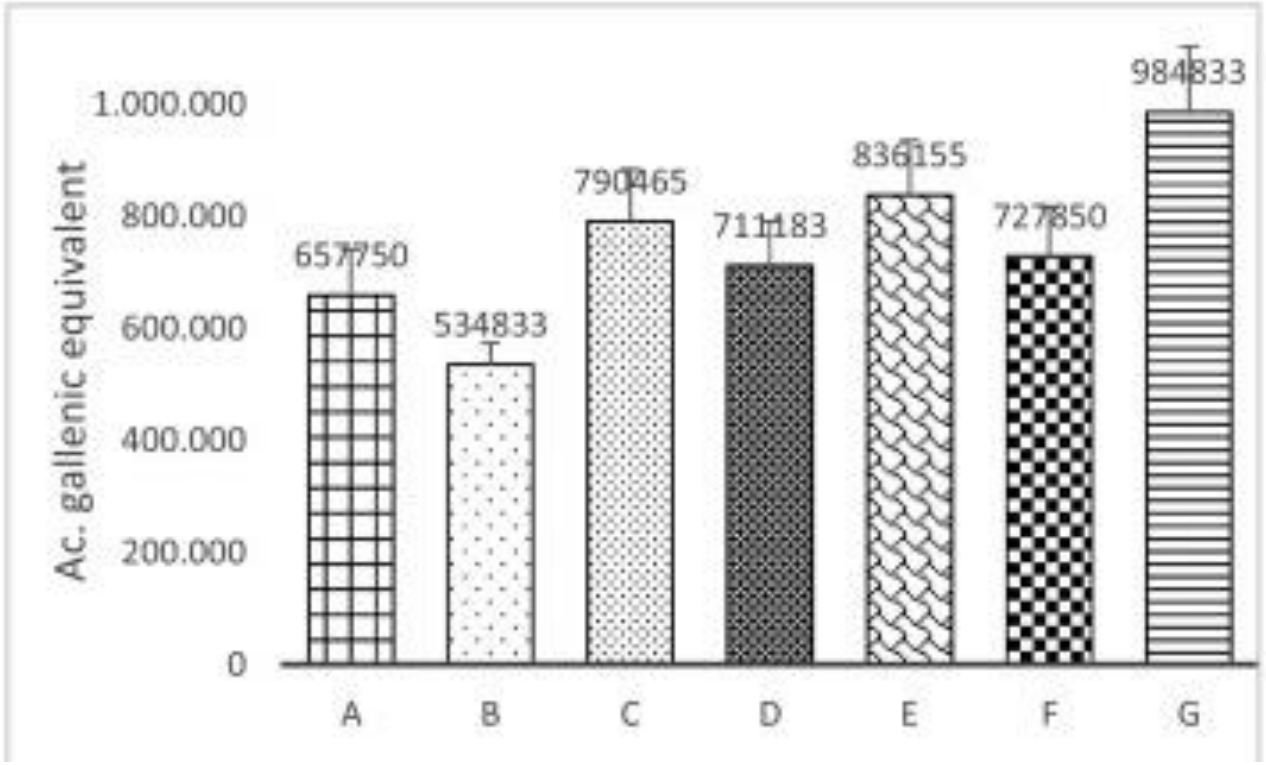


N Engl J Med 2021;384:808-817

Not all albumins are equal



Alterations in the albumin molecule decreasing efficacy in circulating albumin and albumin products



Antioxidant activity of different commercial albumin solutions

Naldi et al Structural and functional integrity of human serum albumin: Analytical approaches and clinical relevance in patients with liver cirrhosis, Journal of Pharmaceutical and Biomedical Analysis, Volume 144, 2017, Pages 138-153,

Mori et al Post-translational modifications and antioxidant properties of different therapeutic human serum albumins, International Journal of Biological Macromolecules, Volume 183, 2021, Pages 927-935,

A call to arms

We need to

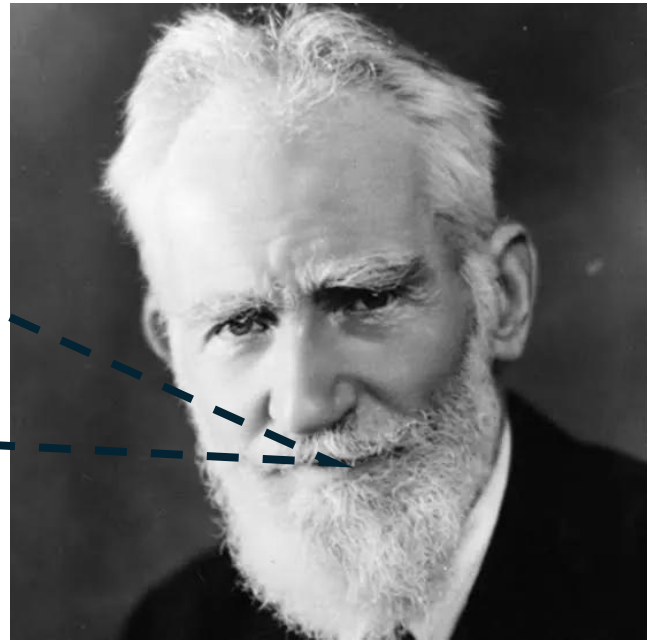
- Understand the indications
- Generate evidence for usage, dosage…
- Optimise procurement processes
- Ensure appropriate standards
- Examine and encourage alternatives

Through

- FOLLOWING THE DATA
- IGNORING POPULISIM
  - ⊗ “MORE PLASMA”
  - ⊗ “OFF LABEL USE”
  - ⊗ ETC ETC
- PRACTICING EVIDENCE BASED MEDICINE
  - EFFICACY IS NOT EFFECTIVENESS

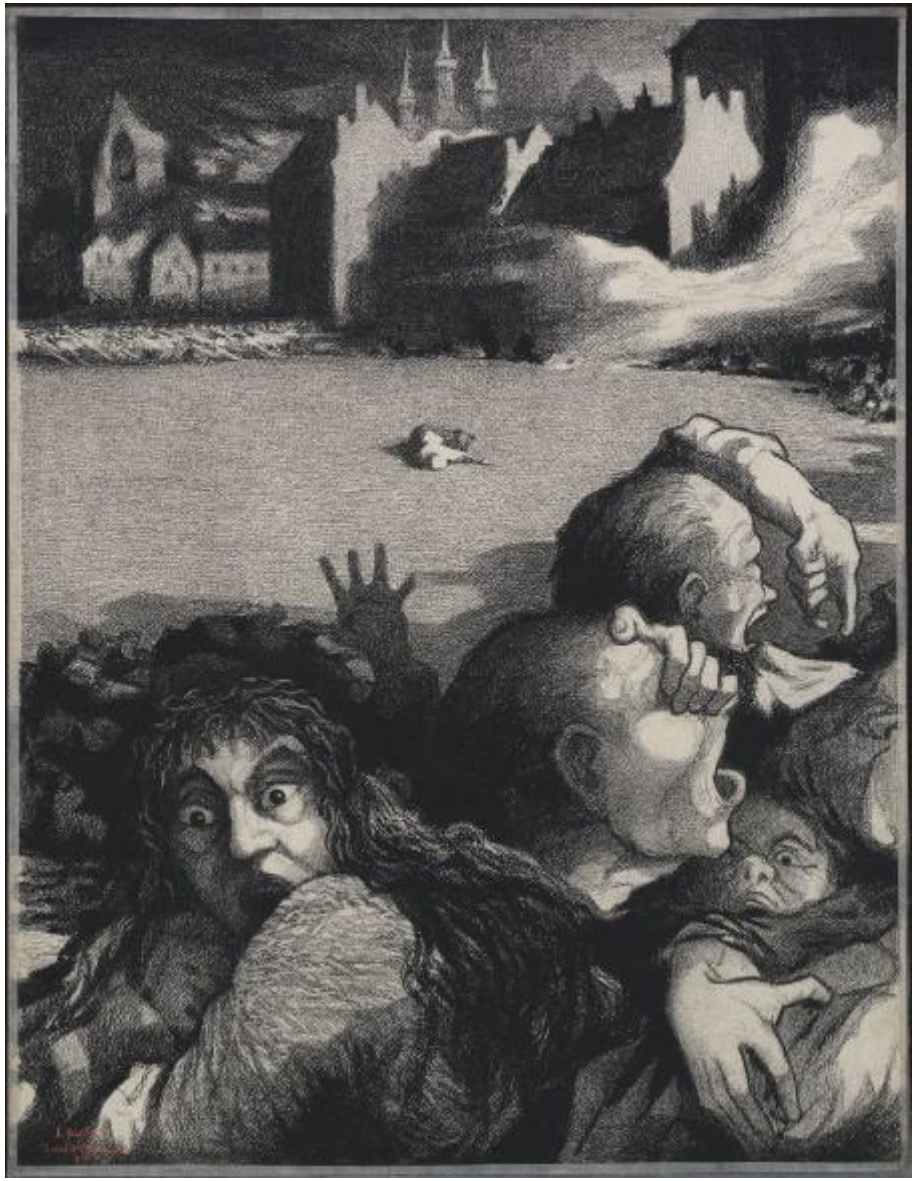
None of this is easy – BUT

"Life is not meant to be easy, my child; but take courage: it can be delightful."



This talk is dedicated to the memory of the 248 citizens of Leuven, who, on the 25th of August 1914, were murdered by the soldiers of a neighbouring and more powerful nation, led by an autocratic megalomaniac, which twice in the last century, invaded Belgium in breach of international treaties. They sacked the town and burned down the historic library.

Any similarities to ongoing events are entirely intentional.



Louvain, by Belgian artist Gisbert Combaz (1916)