GBS | CIDP
Foundation International

SUPPORT • EDUCATION • RESEARCH • ADVOCACY

The need for plasma beyond PIDs

A patient's perspective

Nancy Di Salvo
GBS|CIDP Foundation International and EU
Vice President of International Affairs
Board of Directors
Italian Liaison

*November 1990 (at **age 20**):*

- ***GBS** (Guillain Barrè Syndrome) diagnosis*
- *100 days of hospitalization, treated with corticosteroids*
- *It took over 2 months to take my first steps with a walker, 5 months to walk (with crutches)*
- *After 16 months: hospitalized again with the diagnosis of **GBS relapse***
- *Treatment then was **intravenous immunoglobulin***
- *Released from the hospital after "only" 12 days*
- *Constantly getting better and reacquiring my physical ability*

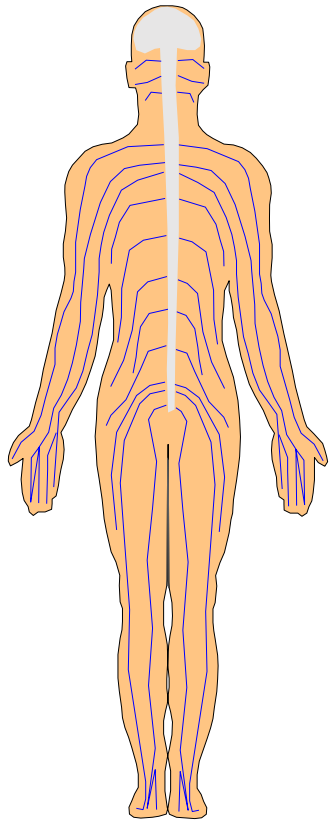
- *After 15 years (2005): weakness and difficulty with motor skills*
- ***Diagnosed with CIDP in 2006***
- *Treated with intravenous immunoglobulins every month, 3 to 5 days a week in day hospital.*
- *Slowly recovered my ability to stand, to walk without crutches*
- *Regained a life that is not focused on my disability but on my goals*
- *Subcutaneous treatment with immunoglobulins better fit my fulltime job and improved stability*
- *Immunoglobulins provide a better **quality of life!***

GBS | CIDP
Foundation International

SUPPORT • EDUCATION • RESEARCH • ADVOCACY

What is CIDP and the related disorder MMN?

- ✓ Disorders of the peripheral nervous system
- ✓ Immune-mediated
- ✓ Damage to myelin sheath and/or axon
- ✓ Effective treatment = **immunoglobulins**



GBS | CIDP
Foundation International

SUPPORT • EDUCATION • RESEARCH • ADVOCACY

CIDP ***Chronic Inflammatory Demyelinating Polyneuropathy***
MMN ***Multifocal Motor Neuropathy***

- Both chronic, slowly progressive
- CIDP: Muscle weakness (paralysis), symmetrical, sensory symptoms, pain
- MMN: Muscle weakness (paralysis), non symmetrical
- Prevalence: CIDP 28 per 1M, MMN 10 per 1M
- Long-term maintenance treatment with immunoglobulins required

What if CIDP and MMN are left untreated or are insufficiently treated?

- ❑ Progression of disease => permanent Nerve damage => severe/permanent disability

  e.g.: loss of hand function, wheelchair-dependency

- ❑ Alternatives to immunoglobulins?

  - CIDP: Yes, corticosteroids or plasma exchange (*but not as a long-term option and with many side effects*)
  - MMN: No other treatment

CIDP and MMN:

- Affect previously healthy and active people
- Without proper treatment, prevent them to participate in and contribute to society => financial/economic consequences for patient and society!
- Untreated, hugely impact patients' quality of life, as Ig treatments are indispensable to regain and maintain function and therefore QoL!

GBS | CIDP
Foundation International

SUPPORT • EDUCATION • RESEARCH • ADVOCACY

What matters to patients with CIDP and MMN:

- Product safety: The EMA has guaranteed product safety, maintaining high standards.
- Availability: Lack of access
  - *Will there be enough IG for the current demand?*
  - *Will patients have access to IG treatments?*
- Affordability: The cost of the treatment is not always sustainable for those that can't rely on a valid government health system.
- Access issues: The causes vary per country and that should be unified within the EU

Access issues endanger patients' safety and QoL

GBS | CIDP
Foundation International

SUPPORT • EDUCATION • RESEARCH • ADVOCACY

Immunoglubuline treatment makes it possible for me to:

- Walk unaided
- Climb stairs
- Have a fulltime job
- Shop for groceries
- Travel
- Live with less pain
- Have a social life
- Volunteer in my Franciscan group
- Volunteer as a patient advocate
- Take care of my family

GBS | CIDP Foundation International

SUPPORT • EDUCATION • RESEARCH • ADVOCACY

In conclusion

- Patients' access to this indispensable and unique therapy must be ensured
- As the need for immunoglobulins will only increase in the future, so will the need for plasma as starting material, for it takes approximately **130 plasma donations** to produce one year's supply of immunoglobulin for an adult with PI.
- Additional pressure has been added due to Covid-19
- Collective focus must be on increasing Europe's plasma collection in order to ensure availability while reducing Europe's reliance on US plasma!

Thank you for your attention!

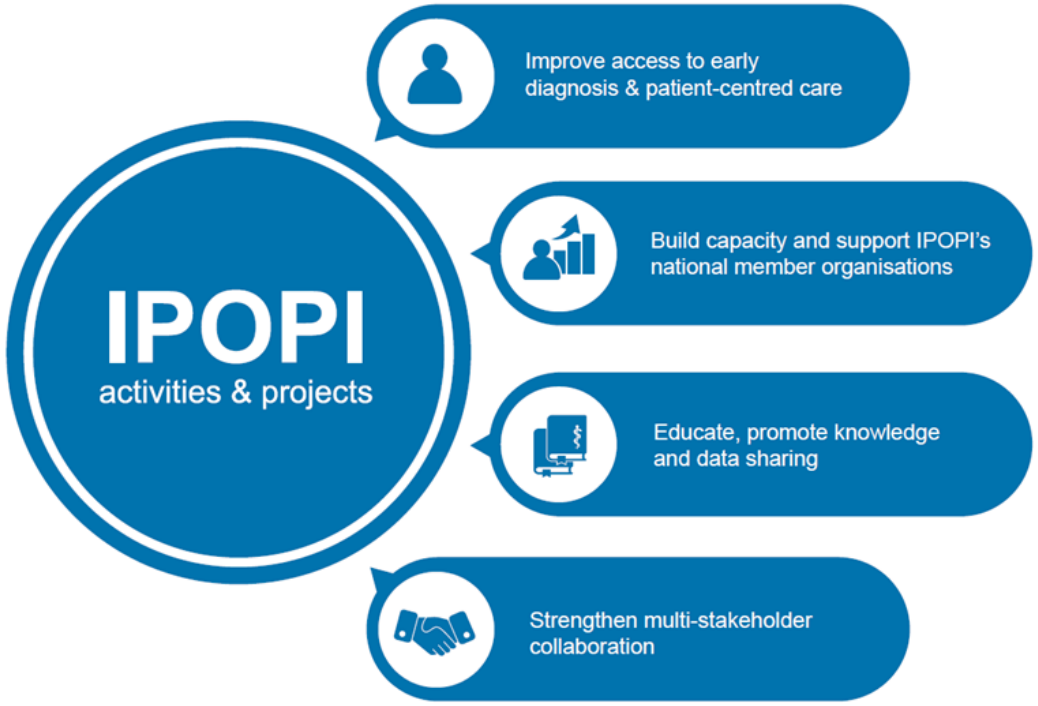
Setting the scene:
Why plasma collection matters so much to patients with PIDs

Johan Prevot
Executive Director, IPOPI

22nd EU PID Forum
European Parliament (Brussels), 16 January 2023

Introduction to IPOPI

- The association of national patient organisation dedicated to improving:
  - Awareness
  - Access to early diagnosis
  - Access to care
- For patients living with primary immunodeficiencies (PIDs), worldwide



ecdc
EUROPEAN CENTRE FOR DISEASE PREVENTION AND CONTROL

- Supporting organisation/speaker at the European Antibiotic Awareness Day (EAAD)
- COVID19 & SoHo dossier

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

- Member of the Patients and Consumers Working Party (PCWP)

World Health Organization

- Essential Medicines Lists
- ICSPP

unitar
United Nations Institute for Training and Research

- Strengthening healthcare systems to meet patients' need for plasma and plasma-derived therapies

IPOPI
INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

Primary Immunodeficiencies

- 480 rare and chronic diseases
- The immune system does not work adequately or at all
- Affect children and adults
- Depending on the PID, patients can have:
  - Opportunistic infections (pneumonia, bronchitis, sinus & ear infections, meningitis, skin infections)
  - Persistent inflammation of internal organs (lungs, nervous system, heart…)
  - Autoimmunity (lupus, rheumatoid arthritis, type-1 diabetes)
  - Delayed growth and development
  - Blood disorders (low platelet count or anaemia)
- Life-impairing and life-threatening life long conditions

IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

Treating patients with PIDs

KEY for a majority of PID patients

- Anti-infectious therapies
  - antibiotics,
  - antiviral,
  - antifungal,
  - antiparasitic

- Other supportive therapies
  - thrombopoietin receptor agonists,
  - C1 inhibitor concrentrate,
  - growth factors,
  - cytokines/interleukins,
  - monoclonal antibodies,
  - immunosuppressors and immunomodulators,
  - enzyme replacement therapy for ADA SCID)

- **Immunoglobulin replacement therapies**
  - **Intravenous**
  - **Subcutaneous**

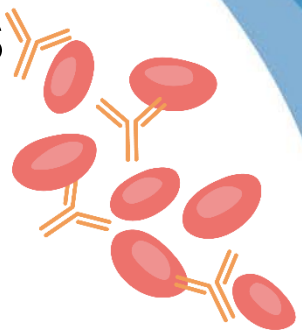
- Vaccines

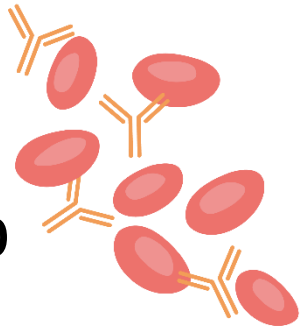
- Curative treatments
  - Hematopoietic stem cell transplantation
  - Gene therapy
  - Thymic transplant

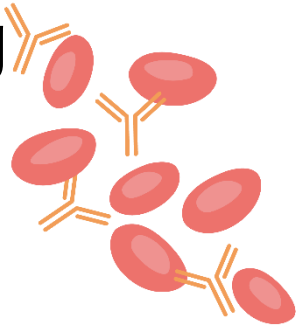
IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

Immunoglobulin (IG) replacement therapies

- For PID patients, Igs REPLACE what their immune system is missing to fight foreign microorganisms (viruses, bacteria...)
- No alternative treatments to Ig therapy.
- No single Ig works for all patients with PIDs → choice of therapies is key
- Igs are needed life-long
- Igs are developed from human plasma. No alternative way to produce them
- Patients in the EU and the rest of the world have been facing challenges in their access to Igs.







IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

Plasma for the development of Igs & other therapies

- One PID patient needs 130 donations of plasma a year to stay alive
- Plasma can be collected from whole blood donations (recovered plasma) or directly through plasmapheresis (source plasma) – **plasmapheresis allows to collect more, more often**
- The demand for IG therapies has been increasing steadily for a number of years.. new conditions, earlier diagnosis…
- EU patients rely heavily on therapies developed from plasma from:
  - United States (not enough plasma collected in Europe)
  - 4 EU member states: Germany + Austria + Czech Republic + Hungary collect significantly more than other EU member states

The main safety concern for patients with PIDs in the EU is SUPPLY

Continued and stable access to Igs as prescribed by the treating physician.

IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

What patients with PIDs need from the SoHO legislation? (1)

- **The EU needs to significantly collect more plasma**
- Plasma collection worldwide should be more regionally balanced.
- Plasmapheresis is key to collect more
- Current patient needs for plasma products can only be met with the input of both the public and private sectors
- Information to donors & general public is important but not enough.

What patients with PIDs need from the SoHO legislation? (2)

- **Policy and guidelines based on scientific evidence and facts.**
- Voluntary & unpaid donation allowing for compensation / reimbursement of expenses and inconveniences.
- Exchange of best practices at EU level supported by the European Commission is key to ensure sustainable and resilient supply of SoHOs.
- Swift implementation of crisis preparedness obligations and development of SoHO emergency plans in consultation with patients

The EU has the opportunity through this regulation to improve plasma collection so patients in need have access to their therapies

IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

PLUS stakeholders meeting 24-24 January 2023 – united for the patients!

alpha-1Plus BELGIUM

eba EUROPEAN BLOOD ALLIANCE

EHC european haemophilia consortium

EPA European Plasma Alliance

GBS|CIDP Foundation International

FIODS

IFPA international plasma fractionation association

IPOPI INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

PPTA Plasma Protein Therapeutics Association

IPOPI INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

PLUS PLATFORM OF PLASMA PROTEIN USERS

Agreements on key aspects of Soho regulation at PLUS stakeholders meeting

- Plasma as implying a “significant risk” (recital 13):
  - Agreement: As currently phrased this sentence could be subject to misinterpretation and could become counterproductive especially in a context needing to collect more plasma in the EU

- Voluntary unpaid donations contribute to high safety of Soho (recital 18)
  - Agreement: Plasma for fractionation from remunerated and non-remunerated donors results in PDMPs of equivalent safety

IPOPI INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

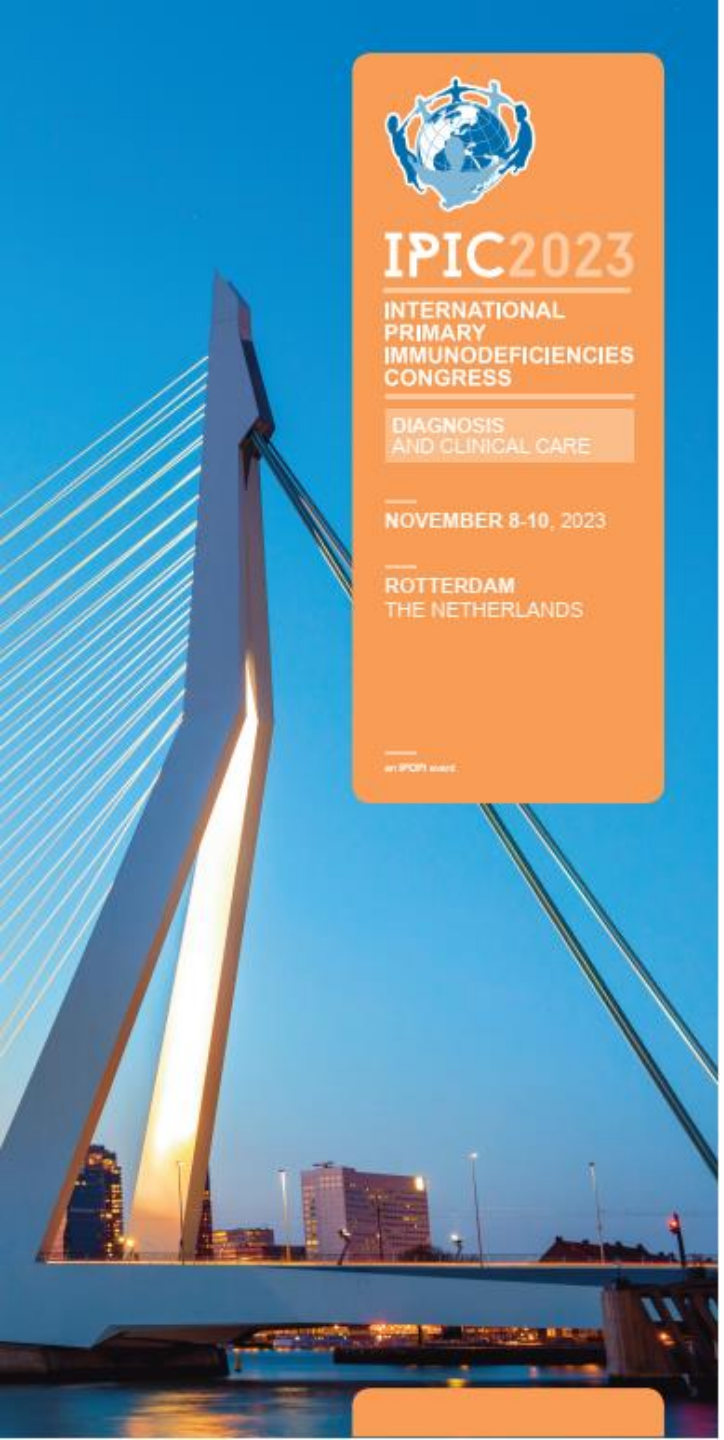
PLUS PLATFORM OF PLASMA PROTEIN USERS

Requests for new recitals and articles from PLUS stakeholders meeting

- It is important that the Commission, the ECDC, the EDQM, when assessing scientific guidelines **involve, when appropriate, existing professional, scientific, industry, donor and patient representative groups in the field of SoHOs**
- **Member States are encouraged to develop or strengthen plasmapheresis** programmes to ensure capacity to collect more plasma and the Commission shall assist them in this task by providing guidance and facilitating the **exchange of best practices**.
- The Commission shall, within two years of the adoption of this Regulation, **publish a strategy for promoting greater European autonomy in the supply of SoHO**. This strategy will set specific targets for SoHO, as defined by the Commission in coordination with national competent authorities, the European Parliament, and relevant professional, scient industry, donor and patient representative groups.

IPOPI INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

PLUS PLATFORM OF PLASMA PROTEIN USERS



Thank you for your attention!

EUROPE'S GROWING NEED FOR PLASMA

Matthew Hotchko, PhD
Marketing Research Bureau

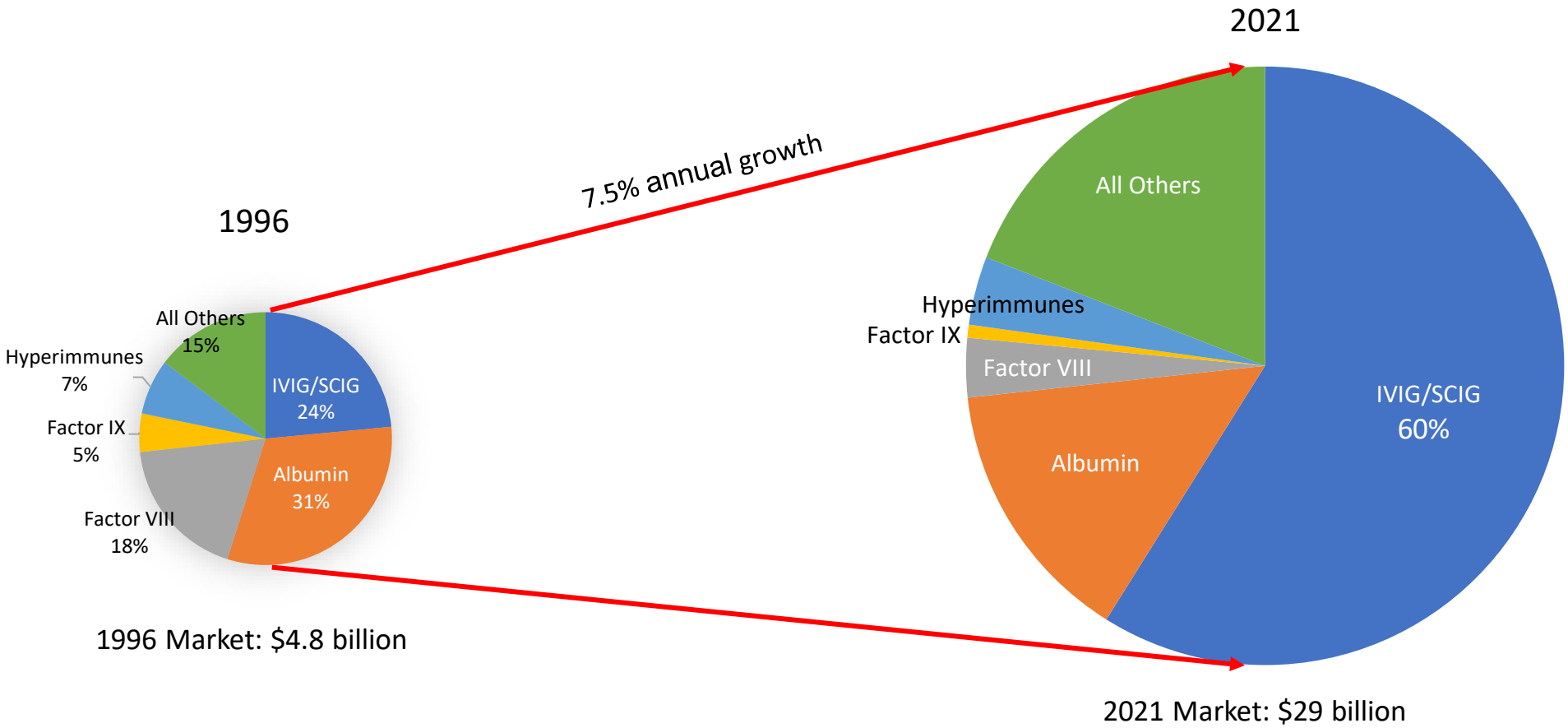
IPOPI 22nd EU PID forum
January 26, 2023

METHODOLOGY AND DISCLAIMER

The data used to develop the charts and tables shown in this presentation have been compiled from surveys conducted by the Marketing Research Bureau in over 90 countries and published in various syndicated reports.

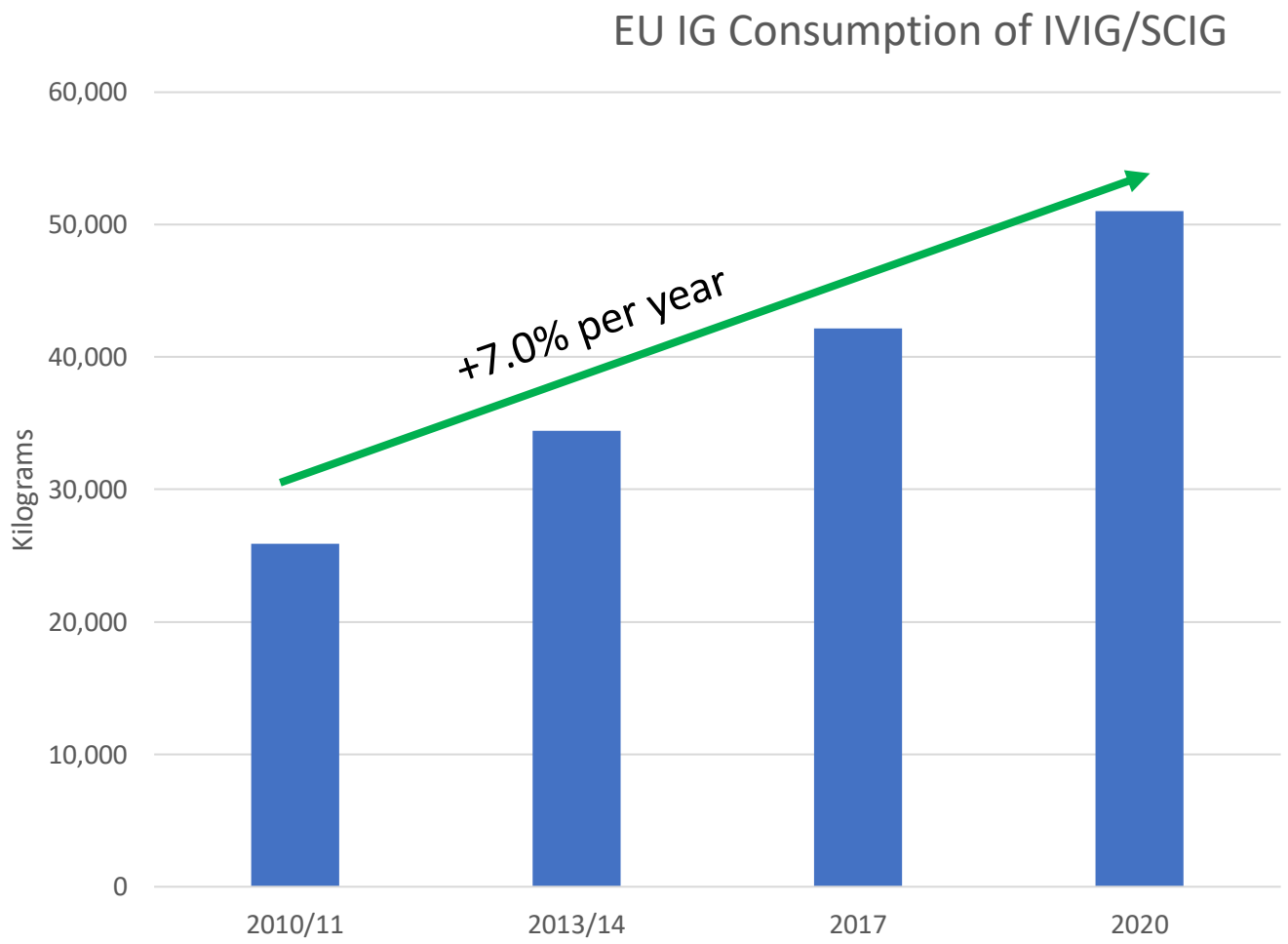
All the data and information originate from sources generally available to the public. Their accuracy is not guaranteed, and the Marketing Research Bureau assumes no liability for their use.

Immunoglobulins (IVIG & SCIG) have dominated the plasma industry over the past 25 years due to growing patient needs



Note: Pie charts are drawn to scale

IG consumed by patients in the EU shows strong growth up to the start of the COVID19 pandemic

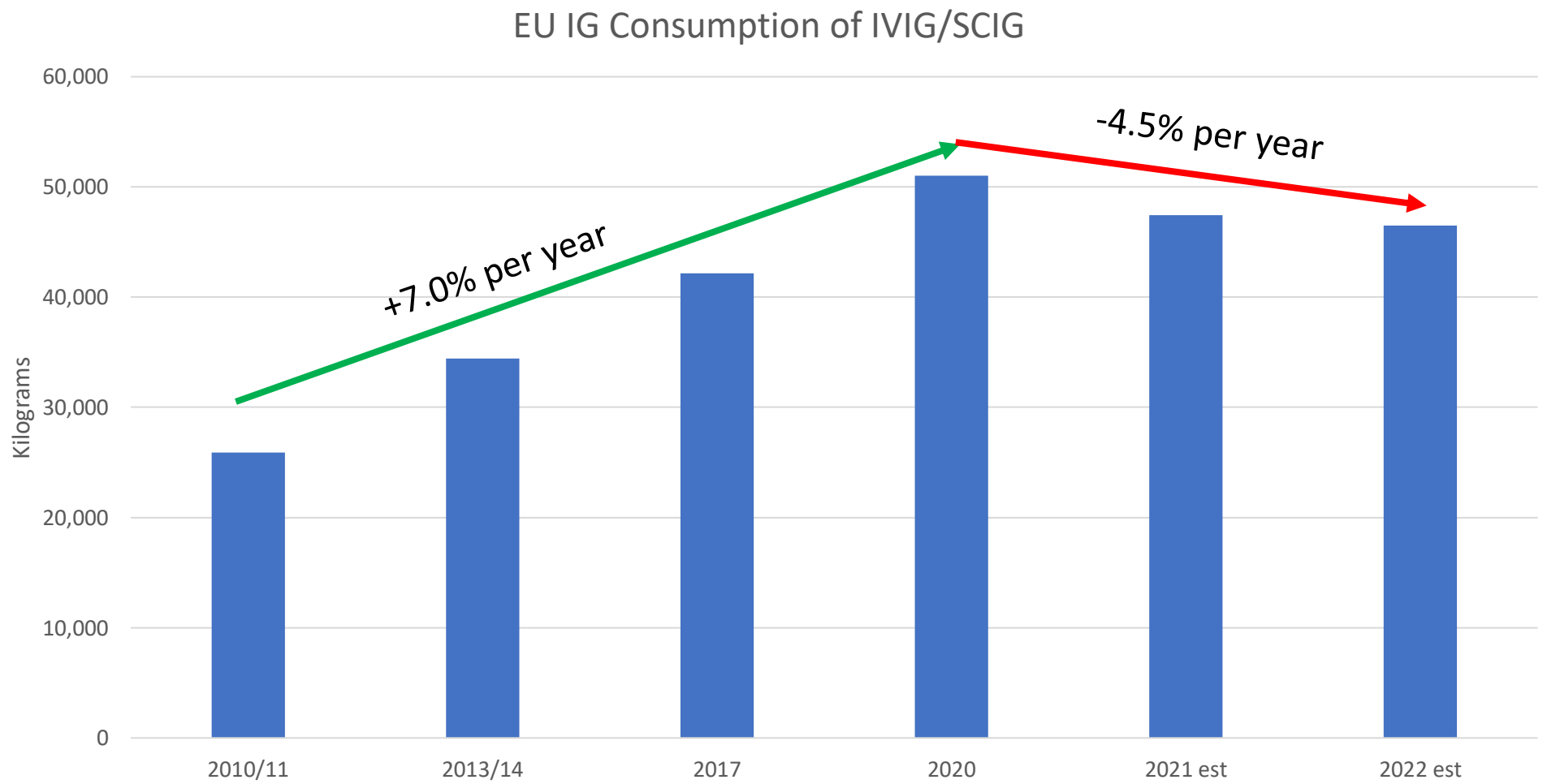


<u>IG Growth was driven by:</u>

- More diagnosis and usage by patients with primary immunodeficiencies
- Neurology growth such as CIDP and many other autoimmune conditions
- Increase in usage of immunosuppressants leading to secondary immunodeficiencies
- Increased reimbursement of the therapy in many countries due to medical evidence of patient benefit

*EU minus United Kingdom to be consistent over time

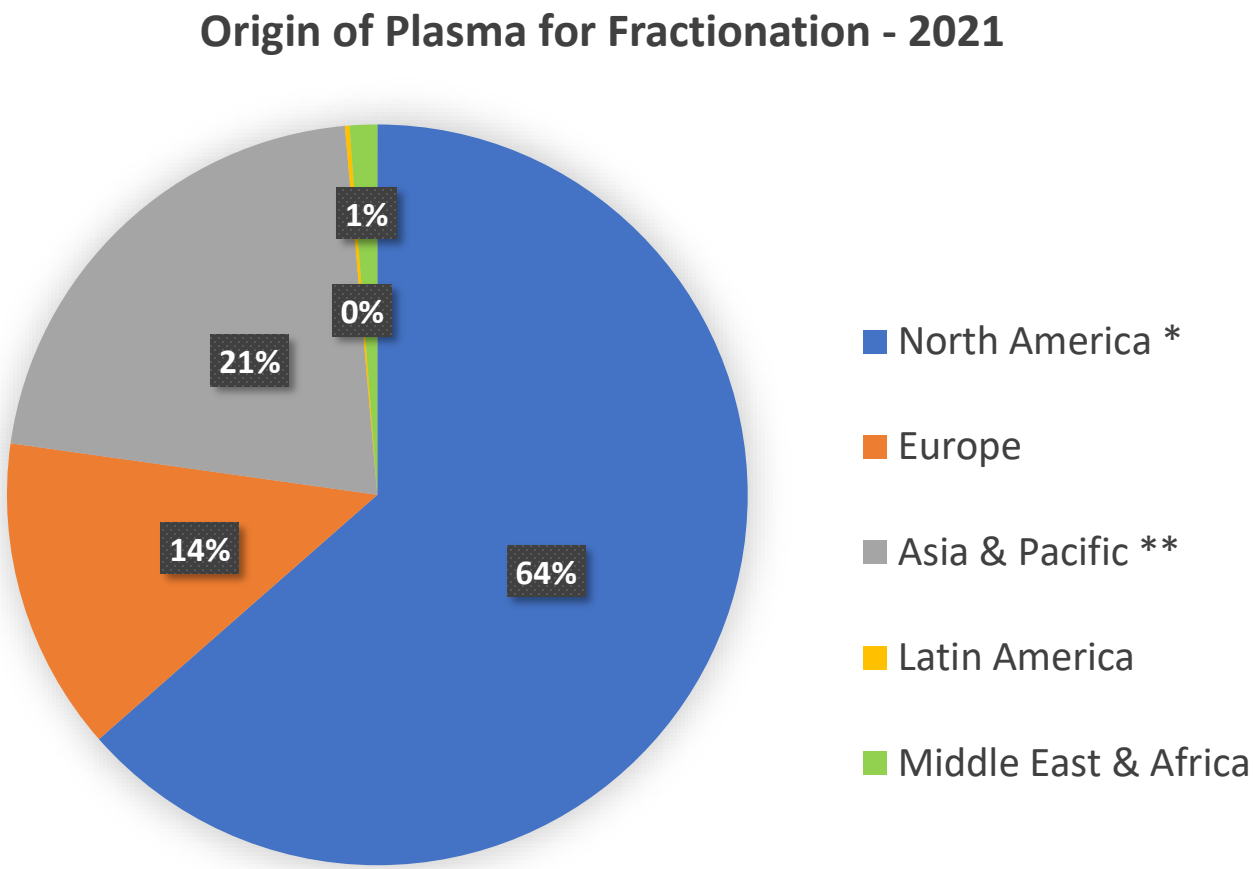
The COVID19 pandemic caused an acute shortage of product in Europe, leading to lower consumption since 2020



More plasma supply would have resulted in more patients using IG, and continued the historical trend of IG consumption growth due to increased patient need

*EU minus United Kingdom to be consistent over time

How Europe compares to supply of plasma for fractionation

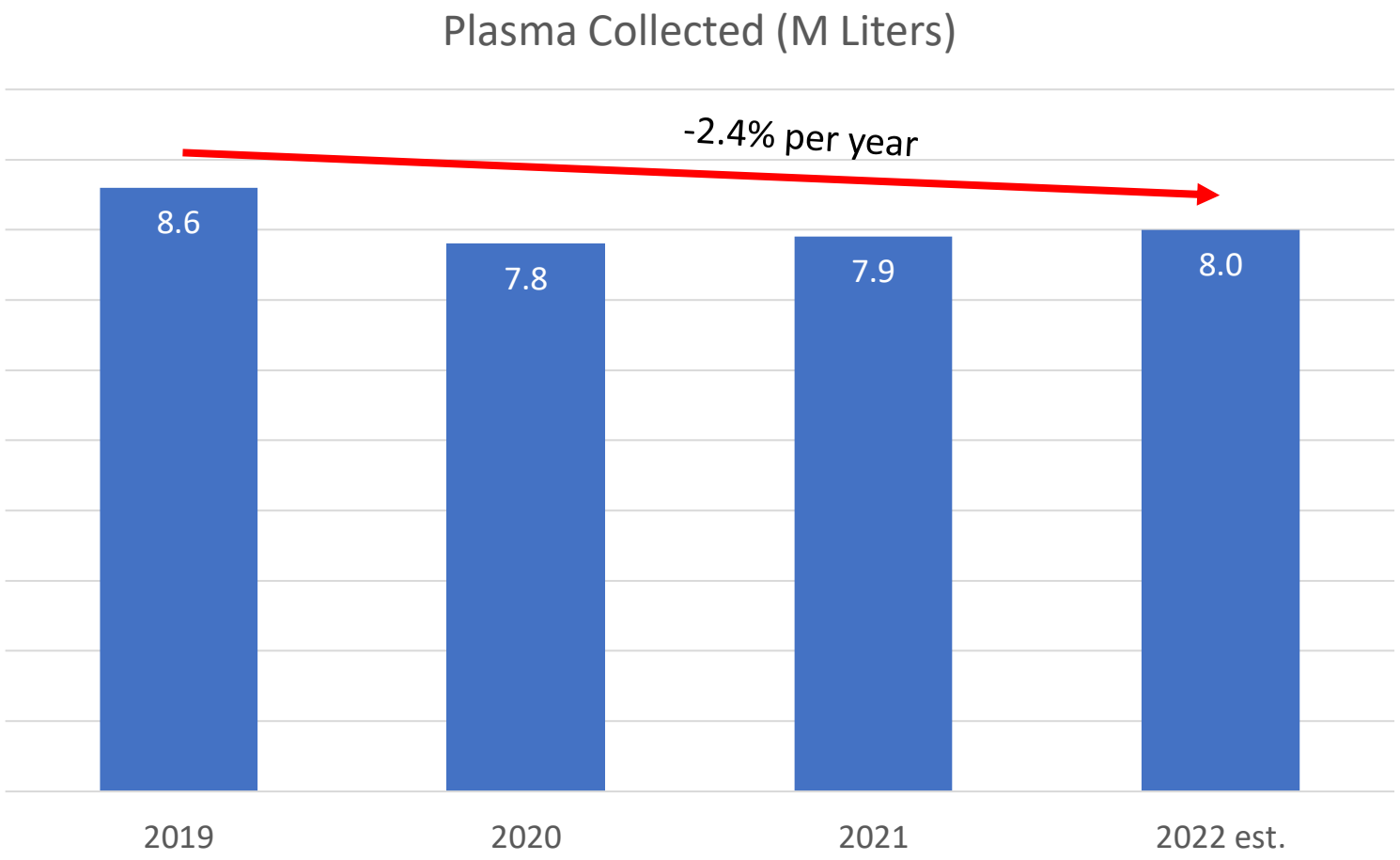


*United States represented 99% of the North America total

** China represented ~70% of Asia & Pacific total

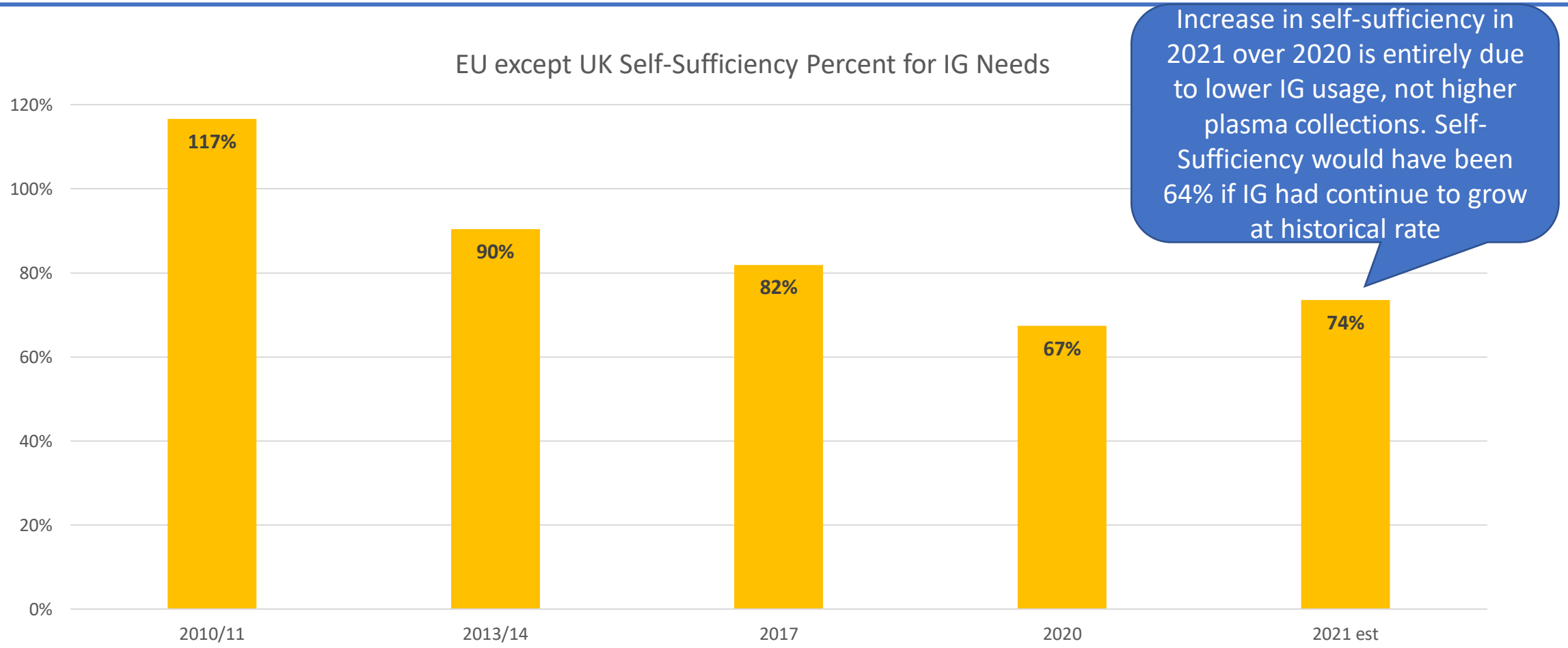
Total Plasma Collection volume 2019: 69 M liters
2020: 59 M liters (-14% vs. 2019)
2021: 61 M liters (-11% vs. 2019)

EU plasma collection from 2019 to 2022 has declined, with just 1% growth since 2020, lead by commercial plasma collection centers



Source (apheresis) plasma has grown over the past few years at 0.5% rate per year, while recovered plasma from whole blood has declined 6% per year. Overall, plasma has declined 2.4% per year the past 3 years on average.

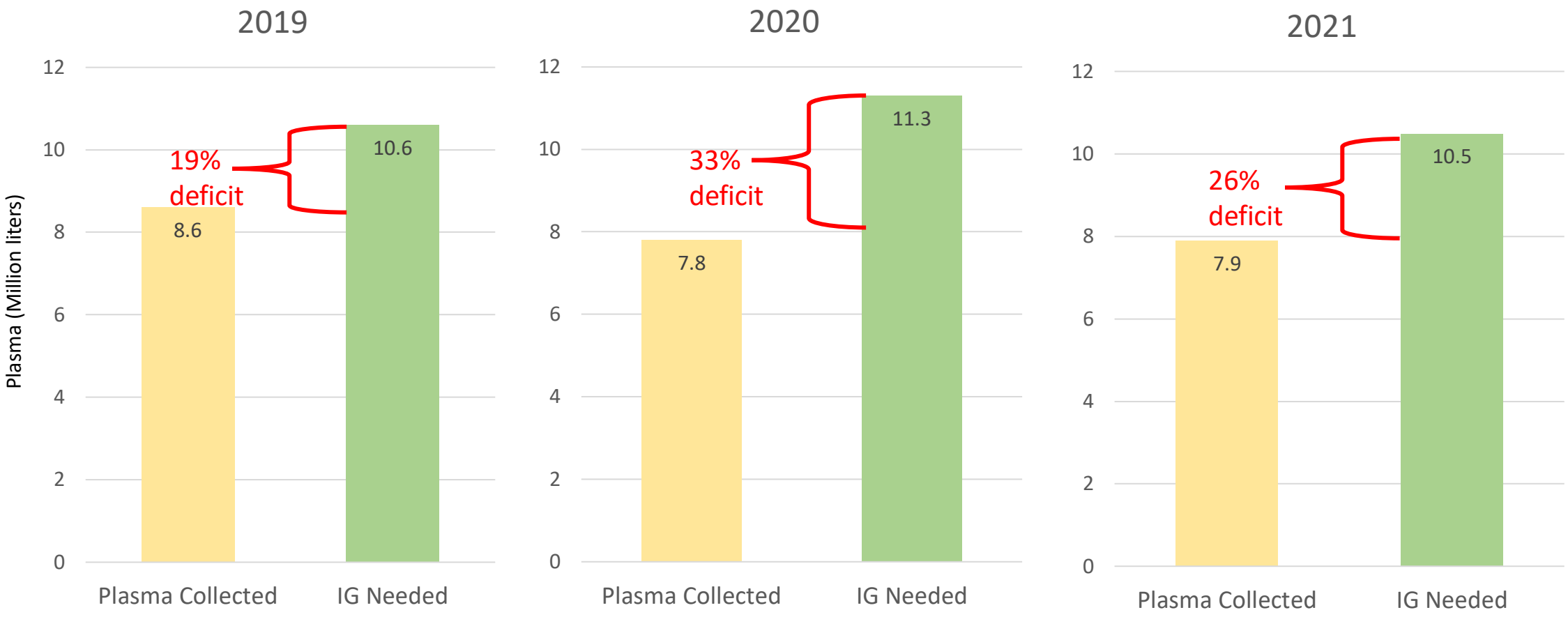
EU* has gone from having a surplus of plasma for IG fractionation to a large deficit, largely due to increased IG needs and slower plasma growth



IG Yield (g/L)	2010/11	2013/14	2017	2020	2021
Recovered	4.7	4.8	4.9	5.0	5.0
Source	3.7	3.8	3.9	4.0	4.0

*EU minus United Kingdom to be consistent over time

2019 to 2021 imbalance of plasma collections and need for IG in European Union



*Assume average blended yield of 4.5 grams IG per liter of plasma

Increase in plasma collections required to meet the IG needs of all patients:

2019: 2.0 million liters
2020: 3.5 million liters
2021: 2.6 million liters
2022: ~2.4 million liters

Suggestions to improve EU plasma supply for fractionation

- ✓European patients will continue to use more IG in the future if it is available due to current demographic and disease trends
- ✓More plasma must be collected in Europe if it is to maintain its current ~75% self-sufficiency level. Even more plasma is needed to improve on this rate for strategic independence of critical medical materials.
- ✓EU should support plasmapheresis programs in all their forms, as they are geared towards plasma for fractionation
  - ✓Support EU funds used to purchase plasmapheresis machines for the collection of plasma
- ✓Support best practices within the Member States by sharing what works best in different countries, and encourage adoption in countries struggling to grow plasma collections

Thank you!

MARKETING RESEARCH BUREAU,

Office Phone: +1-425-502-6265
Email: info@marketingresearchbureau.com
mhotchko@marketingresearchbureau.com

www.marketingresearchbureau.com