GBS|CIDP Foundation International

Guillian-Barré Syndrome

Chronic Inflammatory Demyelinating Polyneuropathy

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Value of Immunoglobulin Products for GBS & CIDP Patients

Jim Crone

GBS/CIDP Patient and

President, Board of Directors,

GBS|CIDP Foundation International

GBS & CIDP 101

- Inflammatory disorders of the peripheral nerves (outside of the brain and spinal cord)
- Usually preceded by an upper respiratory infection, digestive illness, Campylobacter jejuni, or vaccination (rare)
- Autoimmune conditions where the nerve coating, or myelin, is attacked

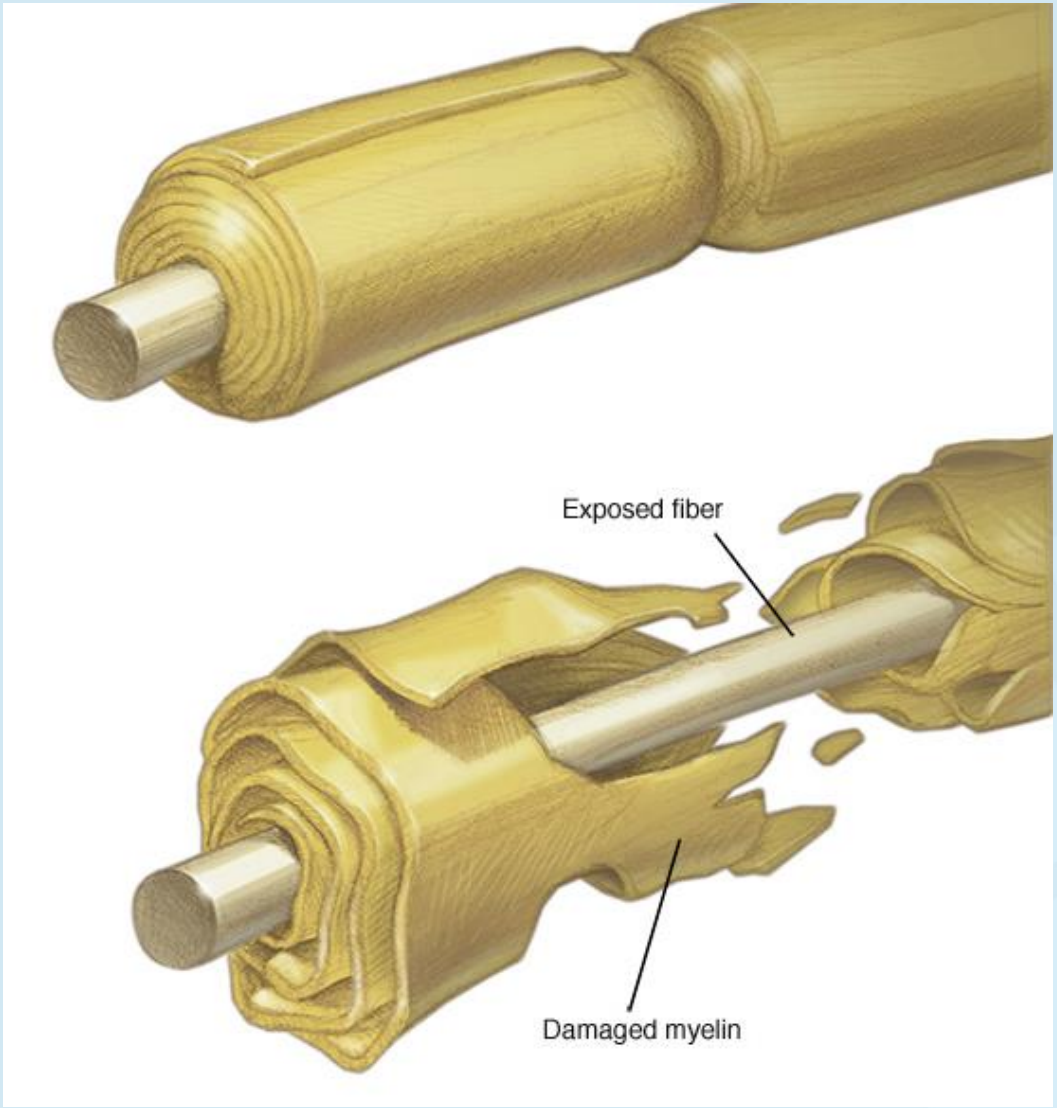

Illustration courtesy Mayo Foundation for Medical Education and Research

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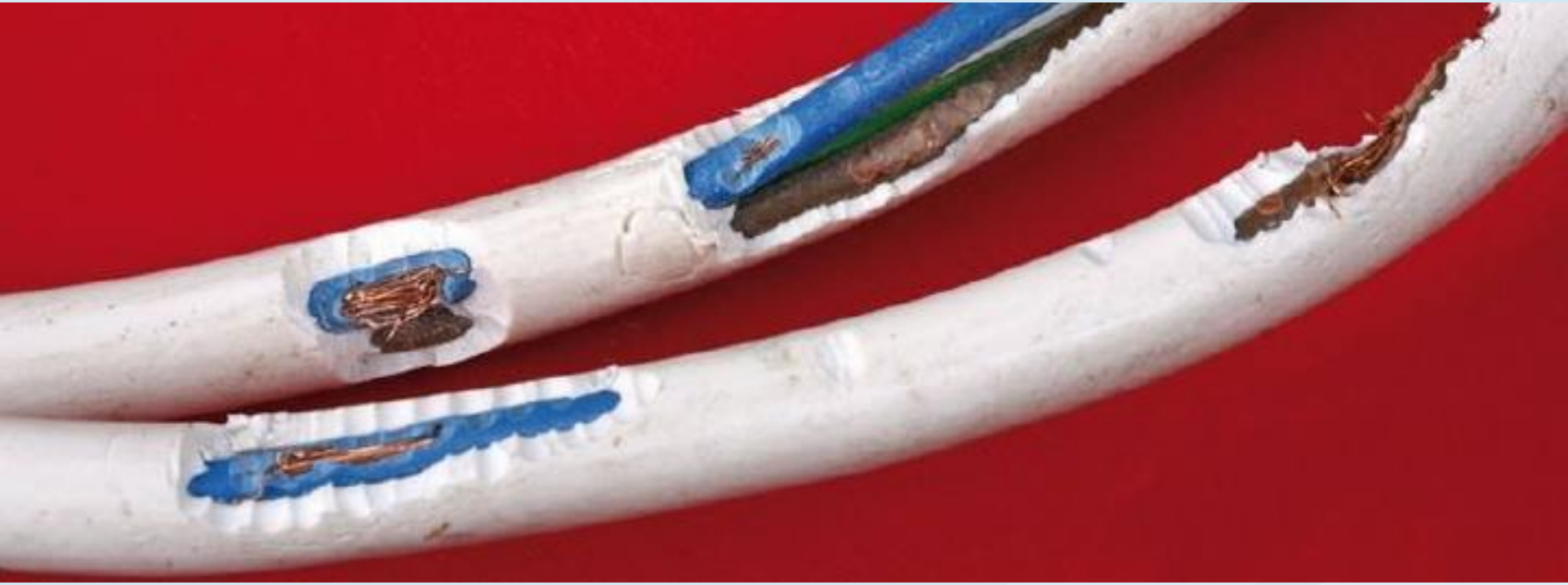


GBS & CIDP 101

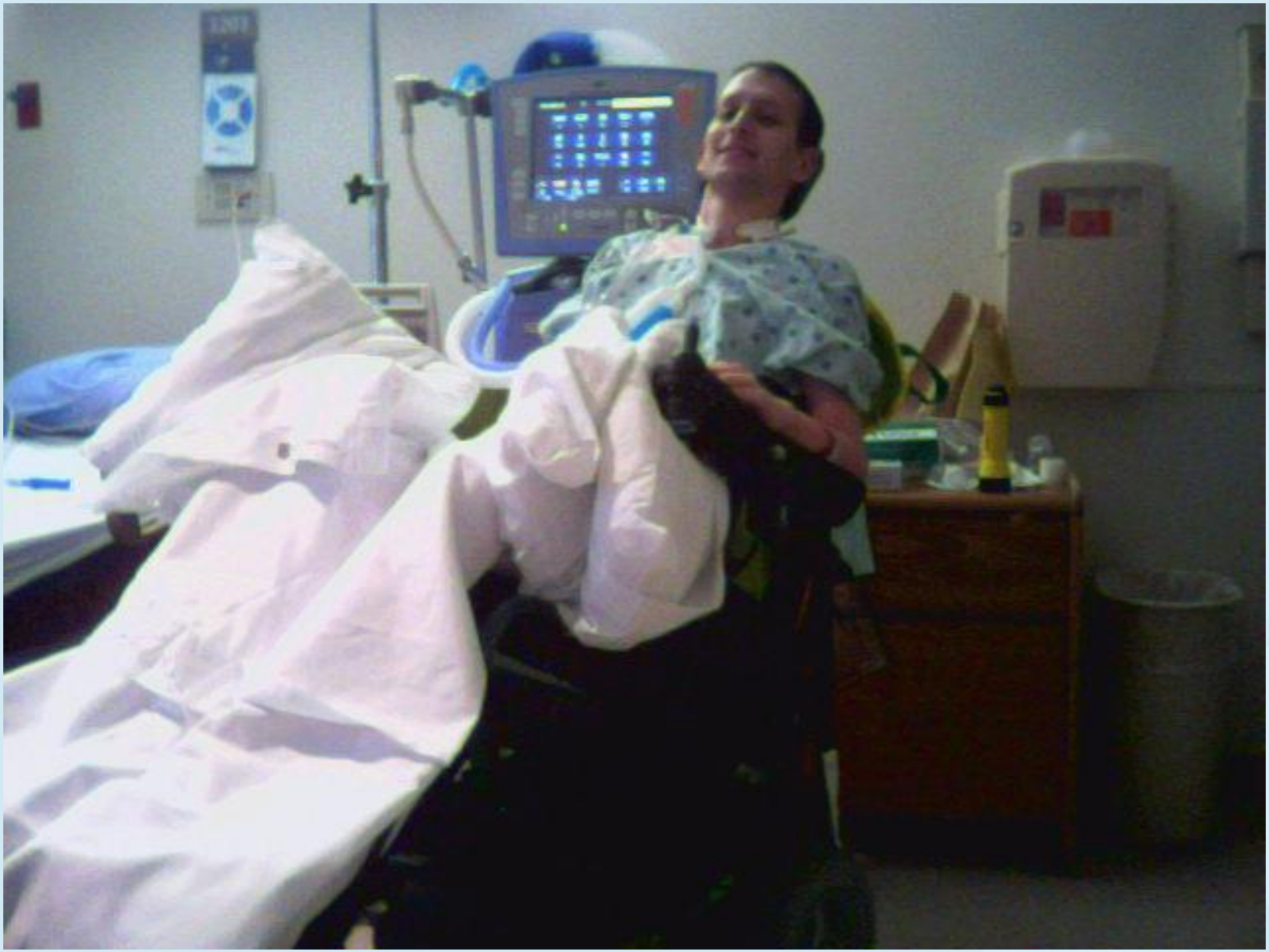
Guillain-Barré Syndrome (GBS)

- Affects 2 in 100,000 individuals
- Acute condition characterized by the rapid onset of numbness, weakness, and often paralysis of the body
- Recovery can take six months to two years or longer; many patients have long-term residual effects
- IVIG or PE treatment used to shorten duration and severity

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

- Affects as high as 9 in 100,000 individuals
- Chronic condition that usually develops over a period or 8 or more weeks with increasing sensory loss and weakness
- Left untreated, 30% of patients progress to wheelchair dependence, but with proper treatment, 90% of patients can walk without aid
- Often requires ongoing treatments of IVIG

My Introduction to Guillain-Barré Syndrome



REHAB

Moving From GBS to a New Challenge – CIDP

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TRANSFUSION

COMMENTARY

Risk-based decision making and ethical considerations in donor compensation for plasma-derived medicinal products

Mark W. Skinner,[1] P. Ann Hedlund Hoppe,[2] Henry G. Grabowski,[3] Richard Manning,[4] Raffi Tachdjian,[5] James F. Crone,[6] and Stuart J. Youngner[7]

For years, policymakers have debated the appropriateness of compensating plasma donors for the manufacture of plasma-derived medicinal products (PDMPs). Recently, the Alliance of Blood Operators (ABO) developed a risk-based decision-making framework for blood safety. In light of these two parallel discussions, now seems to be an opportune time to reanalyze whether an absolutist position against compensation is any longer relevant or would be appropriate if evaluated utilizing a risk-based decision-making approach.

Meeting the health needs of patients by providing an adequate supply of safe and effective blood components and PDMPs is the principal goal of blood operators and the plasma industry. Data demonstrate a large and increasing unmet demand for PDMPs worldwide,[1,2] and there is a growing consensus that an insufficient supply of PDMP treatment products is a major safety risk to patients.[3] Accordingly, it has been argued that a "vein-to-vein" approach to risk-based decision making that encompasses patient needs, product safety, ethical treatment of donors, and other ethical issues should be adopted when considering topics like PDMP donor compensation.[4]

In 2010, an International Consensus Conference on Risk-Based Decision Making for Blood Safety noted that, "As blood systems are focusing more on responsible use of health care resources, questions arise as to the most effective way to manage risk at a level that is tolerable and sustainable. Because of the increasing complexity and inconsistency in blood safety decision making, it is timely to explore whether it is possible to create a better decision-making framework based on risk management principles that can be used in various jurisdictions, taking into account social values, ethics, politics, economics, public expectations, and the historical context in which we operate."[4] In conducting the current analysis, we use these findings of the 2010 Consensus Conference and the subsequent framework developed by the ABO to integrate all stakeholder concerns into an overall risk profile to inform the decision-making process (the ABO Framework).[5] In particular, we draw on the two elements of the framework most relevant to our analysis: the assessment component as a refresh of the ethical discussion around compensated plasma donation and the participation strategy for relevant stakeholder engagement that has been missing from past analyses of compensated plasma donation. We note that the economic considerations of compensation for PDMPs have been explored by Grabowski and Manning.[6]

In addition, we draw in part from the analytical structure of the Nuffield Council on Bioethics (Nuffield), which posed the question: How far can society go in its demands on people to act in what many regard as a good cause—that of providing bodily material to benefit others?[7] The welfare of the many patients who do and could benefit from PDMPs is central to our analysis, and the welfare of donors is a powerful complementary consideration.

There are notable distinctions between donor plasma destined for further manufacture into PDMPs and labile whole blood and its components (e.g., red blood cells

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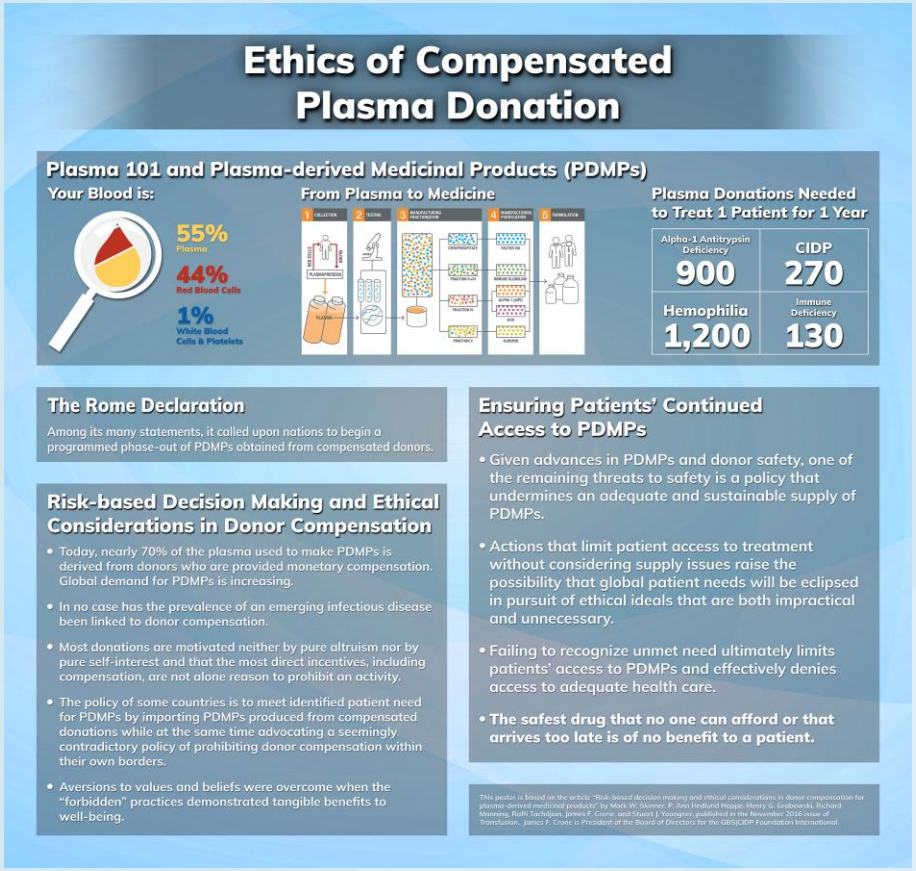


The Need for Plasma

Plasma Donations Needed to Provide One Patient IVIG Treatment for One Year

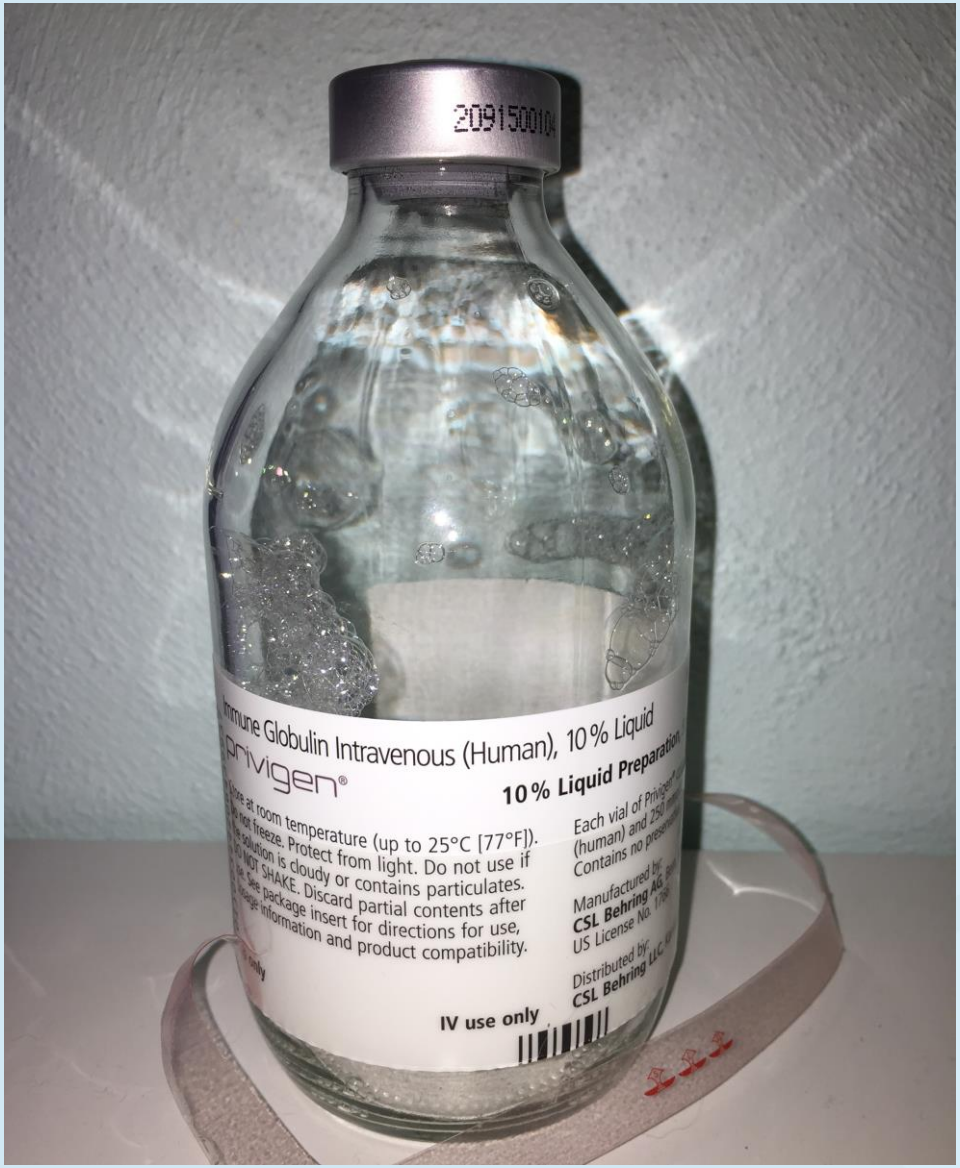
Calculation based on typical dosage, frequency, and average weight

The Need for Plasma

270

Plasma Donations Needed to Provide One Patient IVIG Treatment for One Year

Calculation based on typical dosage, frequency, and average weight


mmune Globulin Intravenous (Human), 10% Liquid
privigen®
10% Liquid Preparation
Store at room temperature (up to 25°C [77°F]).
Do not freeze. Protect from light. Do not use if
the solution is cloudy or contains particulates.
DO NOT SHAKE. Discard partial contents after
Each vial of Privigen
(human) and 250
Contains no preserva
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IV use only

GBS & CIDP Patient Concerns

- Supply of plasma for manufacture of plasma-derived medicinal products (PDMPs)
- Identification of new conditions treatable using IVIG
- Emerging infectious diseases
- Access to healthcare and costs

Thank You!

Jim Crone
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