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Immune Deficiencies Foundation Australia

8th May 2013

POSITION STATEMENT

The Immune Deficiencies Foundation of Australia (IDFA)

Access and choice of Immunoglobulin Therapies for patients living with a Primary Immunodeficiency

This statement is intended to provide a summary of IDFA's position with regards to the importance of ensuring access for patients living with a primary immunodeficiency (PID) to the best suited immunoglobulin (IG) replacement therapy, as selected and prescribed by their physician.

IDFA is concerned by recent developments in several countries that may restrict access to the best suited IG therapy for individual patients. IG therapies are life - saving therapies for PID patients with life-long, chronic conditions. They are listed for the treatment of primary immunodeficiencies on the World Health Organisation (WHO) list of essential medicines. Immunoglobulins are biological therapies derived from human plasma. IG replacement therapy is the most important treatment for a majority of PIDs, as it helps to protect patients against a range of infections and to reduce autoimmune symptoms. IG replacement therapy is used to treat various PIDs, including but not limited to common variable immunodeficiency (CVID), X-linked agammaglobulinaemia (XLA), X-linked hyper-immunoglobulin M (Hyper-IGM) syndrome, Wiskott-Aldrich syndrome (WAS), severe combined immunodeficiency (SCID) and other combined immunodeficiencies.

IG replacement therapy is a lifelong, life-saving treatment which must be administered regularly. There is no alternative treatment to IG therapy for most immunodeficiencies. IG therapies are not generic medicines. Each IG therapy is a unique biological medicinal product and as such IG therapies are not interchangeable. Unlike chemically-based pharmaceuticals, biological medicinal products are composed of an active ingredient derived from a biological source (human plasma in the case of IG therapies). The active ingredients are isolated using complex processes that will have an impact on the properties of the final product.

It is well established that the differences in the processes used to manufacture the products will affect individual patients' tolerability, risk of adverse events, infusion rate, and potential efficacy. Factors such as the volume load, the type and concentration of the excipients used in the preparation, the protein concentration, the osmolarity and osmolality, the pH and the formulation (liquid or lyophilised) will all affect individual patient's tolerability to a given therapy.

In addition the mode of administration in some cases has an impact on how well an individual patient will tolerate a particular IG therapy. Whilst some patients may tolerate well an intravenous product (IVIg) but not a subcutaneous product (SCIg), others may not and vice versa. IDFA promotes patient choice of mode of treatment in consultation with their prescribing physician.

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Lastly it should also be noted that the impact of a poorly tolerated IG therapy will not only affect the patient's health but will bring about significant unnecessary budgetary consequences as the patients will more likely require additional treatments (i.e. antihistamines, extended treatment etc.), thus the importance of ensuring patients receive the best suited therapy to their individual conditions and tolerability profile.

IDFA strongly recommends that necessary measures be taken at national level to ensure PID patients can have continuous and equal access to the widest range possible of safe and effective IG therapies. Prescribing physicians should always have the flexibility to choose the most appropriate therapy for their patients.