Improving the lives of patients with Primary Immunodeficiencies in Africa by providing sustained access to safe immunoglobulin replacement therapies

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The International Patient Organisation for Primary Immunodeficiencies (IPOPI) and the African Society for Immunodeficiencies (ASID) would like to call on African governments to ensure a sustained access to safe immunoglobulin (IG) replacement therapies for African patients with primary immunodeficiencies.

Primary immunodeficiencies (PIDs) are a group of over 380 rare and chronic diseases caused when some components of the immune system (mainly cells and proteins) do not work properly. These deficiencies lead to increased susceptibility to a wide range of infections, and mean that infections can reoccur and leave the individual vulnerable to permanent organ damage, physical disability or even death. PIDs can be treated and, when the right diagnosis and treatment has been given, patients can live normal lives. According to recent publications, it is estimated that there are 902,000 patients with PIDs in Africa, but the real number remains unknown due to lack of awareness, inadequate/poor knowledge amongst medical professionals and lack of access to diagnosis.

Many patients with PID require IG replacement therapy and some will require life-long replacement as their only treatment choice. IG are biological medicines derived from human plasma and help protect immunodeficient patients against infections. When used in much higher doses, IGs can help in the treatment of inflammatory and autoimmune diseases. Currently there is no alternative treatment to IG therapy for most patients with PIDs. Each IG product is unique in its biological medicinal properties and as such the different IG products are not interchangeable. Unlike chemically based pharmaceuticals, the differences in the processes used to manufacture IGs will affect individual patients' tolerability, their risk of adverse events, infusion rate, and the IG potential efficacy.

ASID and IPOPI call on African governments to work, in collaboration with PID experts as well as patients with PIDs and their representatives on the following actions:

1. Facilitate access to immunoglobulin replacement therapies to patients with PID whose lives rely on these therapies.
2. Follow the World Health Organisation list of Essential Medicines for adults and children and include immunoglobulins as replacement therapies in the national lists of essential medicines.
3. Ensure that the national choice and registration of IGs in each country is based on the safety of the products and their compliance with international standards.
4. Provide at least two different types of IG replacement therapies in order to avoid potential shortages and to improve patients' access to a tolerable product.
5. Regulate the use of IG therapies well to avoid the irrational use of these expensive and scarce products.
6. Establish or strengthen a reliable, sustainable and safe blood and plasma collection system in every country.

We join the World Health Organisation in saying that Health for All is possible and that no one should be left behind, no matter how rare the disease is or where the patient comes from. Let’s work together to make it happen!
1. Facilitate access to immunoglobulin (IG) replacement therapies to patients with PID whose lives rely on them.

Health is a human right; everyone should have the information and services necessary to take care of their own health and the health of their families. According to the WHO, "about 100 million people are pushed into extreme poverty each year because of out-of-pocket spending on health". WHO's top priority is to support countries on the path towards universal health coverage, with the aim of ensuring that all people can access the health services they need. This includes access to diagnosis and treatments for people who suffer from rare diseases, such as patients with PID and their IG replacement therapies. Among many other users, patients with PID require access to IG replacement as lifesaving therapies which in most instances would also be needed for life. For all these reasons patients with PID should not have concerns about their access to IG due to their income.

IG replacement therapies have already been recognised as essential by the WHO and other international bodies. It is now time to make them effectively available to patients with PID in the African countries.

2. Follow the World Health Organisation list of Essential Medicines for adults and children and include immunoglobulin replacement therapies in the national lists of essential medicines in African countries.

The World Health Organisation has acknowledged the need for patients with PIDs to have continued access to the treatment that is better adapted to their needs and has included IG in the List of Essential Medicines for adult and paediatric populations. Both lists include immunoglobulin replacement therapies for subcutaneous and intravenous administration.

3. Ensure that the national choice and registration of IGs in each country is based on the safety of the products and their compliance with international standards.

IG therapies undergo a series of purification processes that aim at removing viruses and ensure that the therapy does not transmit any known infectious disease to the patient. Immunoglobulin replacement therapies are developed following the standards set by international regulatory agencies such as the Food and Drug Administration (FDA) or the European Medicines Agency (EMA). Governments should consider investing in IG replacement therapies that are safe and efficient.

4. Provide at least two different types of IG replacement therapies in order to avoid potential shortages and to improve patients’ access to a tolerable product.

The availability of only one IG therapy increases the chances of shortages of these therapies due to unforeseen circumstances (interruption of stock, problems in the production process or site, or distribution challenges). An irregular access to the life-saving IG therapies, not only endangers patients’ health and quality of life, but can leave long-term sequelae requiring additional care. Besides being potentially life-threatening, these health problems will, inevitably, be life-impairing and result in an increase of healthcare and social expenses for the authorities.

Additionally, 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 patient will more likely require additional treatments (e.g. antihistamines, extended treatment, more hospital visits etc); thus the importance of ensuring that patients get the most suitable therapy to their individual conditions and tolerability profile. This also ensures that the patient has a better quality of life, less episodes of ill health and reduced need for additional medication.

5. Regulate the use of IG therapies well to avoid the irrational use of these expensive and scarce products.

Safe plasma is a limited resource and the world’s demand of IG therapies has been steadily increasing over the past 20 years due to several reasons (increased diagnosis, new indications, aging population, 

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1 World Health Organisation Model List of Essential Medicines for adults (link) and children (link) (June 2019).
etc). Countries should be encouraged to develop evidence-based guidelines on the optimal use of IG therapies. This has already been developed by some international agencies or bodies, such as the European Medicines Agency Guideline on Core SPC for human normal immunoglobulin for intravenous administration (IVIg)” (EMA/CHMP/BPWP/94038/2007 Rev. 4), the European consensus proposal for immunoglobulin therapiesiv or the “Asia-Pacific Economic Cooperation Recommendations for enhancing access to safe therapy for persons with immunodeficiency and bleeding disorders”v which has the objective, amongst others, of supporting the efficient use of blood products. At national level, several countries have already developed priority rankings and guidelines for IVIg/SCIg use in clinical practice, including: Australia, Belgium, Germany, Francevi or the UKvii. Based on this evidence PID patients should be prioritized whenever IG distribution and demand management are considered. Additionally, all IG users (doctors, nurses, pharmacists and relevant authorities) should be well informed and trained on the clinical utility and dispensing of the available products to avoid irrational use.

6. Every country should establish and/or strengthen a reliable, sustainable and safe blood and plasma collection system.

IG replacement therapies are plasma-derived medicinal products and, as such, depend on the availability of blood and/or plasma for fractionation. In order to ensure the availability of these scarce resources, national governments should look into establishing or strengthening their blood and plasma collection systems in a reliable, sustained and safe manner. Wastage of plasma should be avoided as much as possible.

iii World Health Organisation. https://www.who.int/campaigns/world-health-day/world-health-day-2019/key-messages