IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

PRIMARY **IMMUNODEFICIENCIES**

WHEN TO GIVE **IMMUNOGLOBULIN REPLACEMENT THERAPY**

ABBREVIATIONS

CVID	Common variable immunodeficiency
IBS	Irritable bowel syndrome
ICF	Immunodeficiency with centromeric instability and facial anomalies
IG	Immunoglobulin
PID	Primary immunodeficiency
SCID	Severe combined immunodeficiency
STAT3	Signal transducer and activator of transcription 3
VODI	Veno-occlusive disease with immunodeficiency

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INTRODUCTION

This booklet explains how you and your physician can decide when to use immunoglobulin replacement therapy

Primary immunodeficiencies (PIDs) are rare diseases that occur when components of the immune system are missing or not working properly, which causes those affected to be more susceptible to infections and other complications than healthy individuals.

Treatment with antibody (also known as immunoglobulin, IG) replacement therapy benefits many individuals who are not able to produce adequate amounts of antibodies. IG replacement therapy products are purified from blood plasma that is donated by healthy individuals. It is carefully screened and has an excellent safety record.

Although this treatment may not prevent all infections, it does usually significantly reduce their frequency and severity in many people with PIDs.

This leaflet provides information on the factors that need to be considered in making this treatment decision.

DECIDING ON IG REPLACEMENT THERAPY: KEY CONSIDERATIONS

IG replacement therapy is recommended for most people with primary B-cell defects and marked antibody deficiencies. In some PIDs, however, other treatments may be more appropriate - it really depends on the type and severity of your PID.

Your physician will be able to provide advice on whether replacement IG therapy is the right option for treating your specific PID

BEFORE STARTING THERAPY

Before starting IG replacement therapy, it is important that your physician completes a number of studies to demonstrate that your IGs are low and that you do not make specific antibodies naturally in response to infections or immunisation with vaccines.

IG levels test

Your physician will use a blood sample to test your immunoglobulin levels, particularly levels of IgG, as well as IgA and IgM.

Vaccine challenge test

A vaccine challenge test provides useful information about how your immune system responds to the vaccine and measures how well you produce IGs. Typically, a vaccine is administered and your immune system's response to the vaccine is measured 4-6 weeks later.

Clinical history

Your clinical history will also be evaluated, for example:

- Antibiotic courses per year
- Weight loss, failure to thrive
- Hospitalisations per 5 years
- Days missed from school/work
- Incidence of various infections and conditions:
  - Pneumonia and upper respiratory infections (including sinusitis)
  - Autoimmune conditions.
  - Sepsis, meningitis, osteomyelitis, empyema, septic arthritis.
  - Splenomegaly or splenectomy.
  - Lymphadenopathy.
  - Infectious diarrhoea.
  - Chronic gastroenteritis, irritable bowel syndrome (IBS)-like disease.
  - Lung function.
  - Bronchietasis.

If your PID is mild and your clinical history is positive, your physician may suggest other treatments before recommending IG replacement therapy, with regular monitoring for any changes in your health. This may include taking low-dose antibiotics for a few months to see how well these protect you from infection.

If your physician does suggest IG replacement therapy, it may be for a trial period of, for example, a year. If you find it beneficial, it may be advisable to continue with the treatment.

The following tables suggest when IG replacement therapy may or may not be useful across a range of PIDs.

IMMUNODEFICIENCIES THAT ALWAYS REQUIRE IG REPLACEMENT THERAPY
Agammaglobulinaemia (X-linked, autosomal recessive or acquired)
Common variable immunodeficiency (CVID)
Good syndrome
Hyper IgM syndrome
SCID before and during bone marrow transplant
Wiskott-Aldrich syndrome

IMMUNODEFICIENCIES THAT MAY REQUIRE IG REPLACEMENT THERAPY

Ataxia telangiectasia
Complement deficiencies (C3, C4 and C5-9), properdin deficiency
Hepatic veno-occlusive disease with immunodeficiency (VODI)
IgG subclass deficiency
Immunodeficiency with centromeric instability and facial anomalies (ICF)
Netherton syndrome
Selective antibody disorder
Severe cases of transient hypogammaglobulinaemia of infancy
Severe combined immunodeficiency (SCID) after bone marrow transplant (some patients recover completely and will not need to continue treatment)
Signal transducer and activator of transcription 3 (STAT3) deficiency
X-linked lymphoproliferative syndrome

IMMUNODEFICIENCIES THAT DO NOT USUALLY REQUIRE IG REPLACEMENT THERAPY

Asymptomatic moderate (even under antibiotic prophylaxis) hypogammaglobulinaemia and normal antibody responses
DiGeorge syndrome
Dyskeratosis congenital
Transient hypogammaglobulinaemia of infancy with severe recurrent infections

FURTHER INFORMATION AND SUPPORT

This booklet has been produced by the International Patient Organisation for Primary Immunodeficiencies (IPOPI). Other booklets are available in this series. For further information and details of PID patient organisations in 52 countries worldwide, please visit **www.ipopi.org**.

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