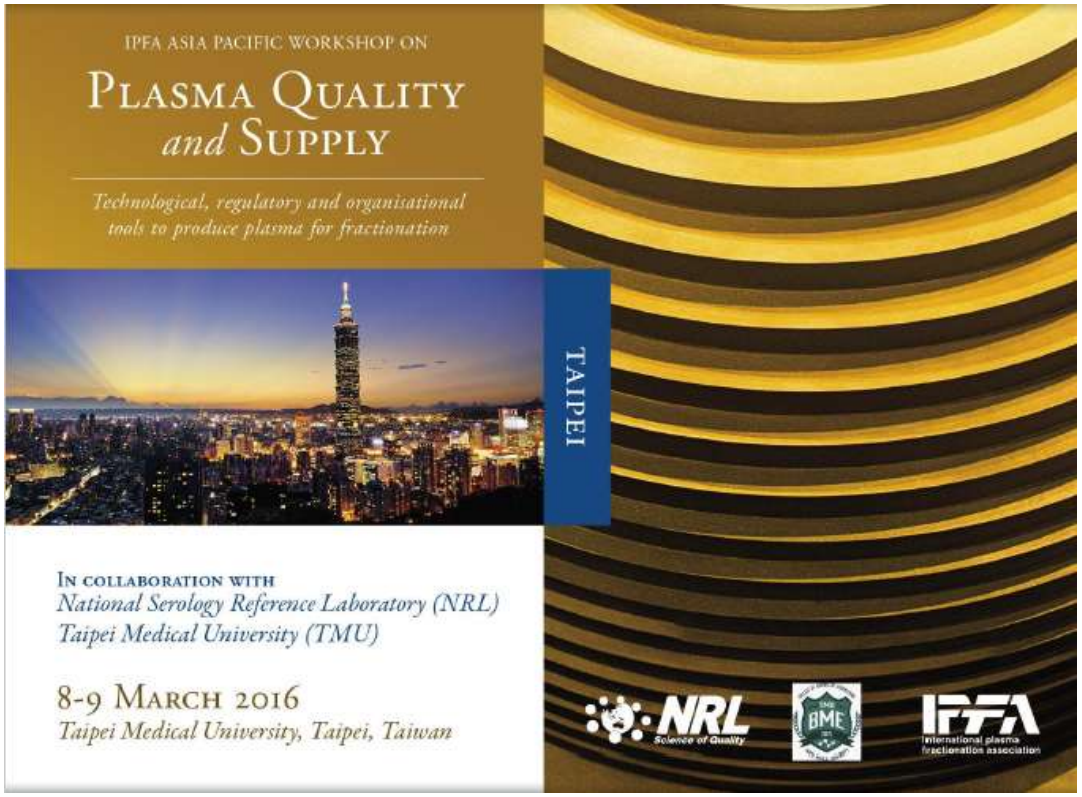
Unmet Needs for Plasma Products
Focus on immunoglobulin therapies for PID patients

Johan Prévot
Executive Director
IPOPI

IPFA Asia Pacific Workshop
Plasma Quality & Supply
Taipei, Taiwan
8 March 2016



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2. Primary Immunodeficiencies
3. Current Treatments and Treatment Unmet Needs
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- IPOPI is the Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide



www.ipopi.org

IPOPI

THE GLOBAL ORGANISATION WORKING
TO **IMPROVE THE QUALITY OF LIFE FOR PEOPLE**
WITH PRIMARY IMMUNODEFICIENCIES



IPOPI's NMOs in Asia



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Objectives



TO IMPROVE:

Awareness

Diagnosis & Care

Number of active Nat. Patient Groups

Stakeholder Collaboration

> for PID patients

(children and adults)

> worldwide





Primary Immunodeficiencies (PIDs)

- Occur in persons born with failed immune systems
- Hereditary or genetic defects,
- Can affect anyone, regardless of age or sex.
- Vary in severity whether one or several parts of the immune system are non functional (innate or adaptive)
- Over 280 different PIDs defined in 9 different groups
- Considered rare disorders but taken as a whole = significant number of patients whose lives are profoundly affected by their condition
- Est. 80% patients no access to adequate care

What do Patients want ?



Not being ill !

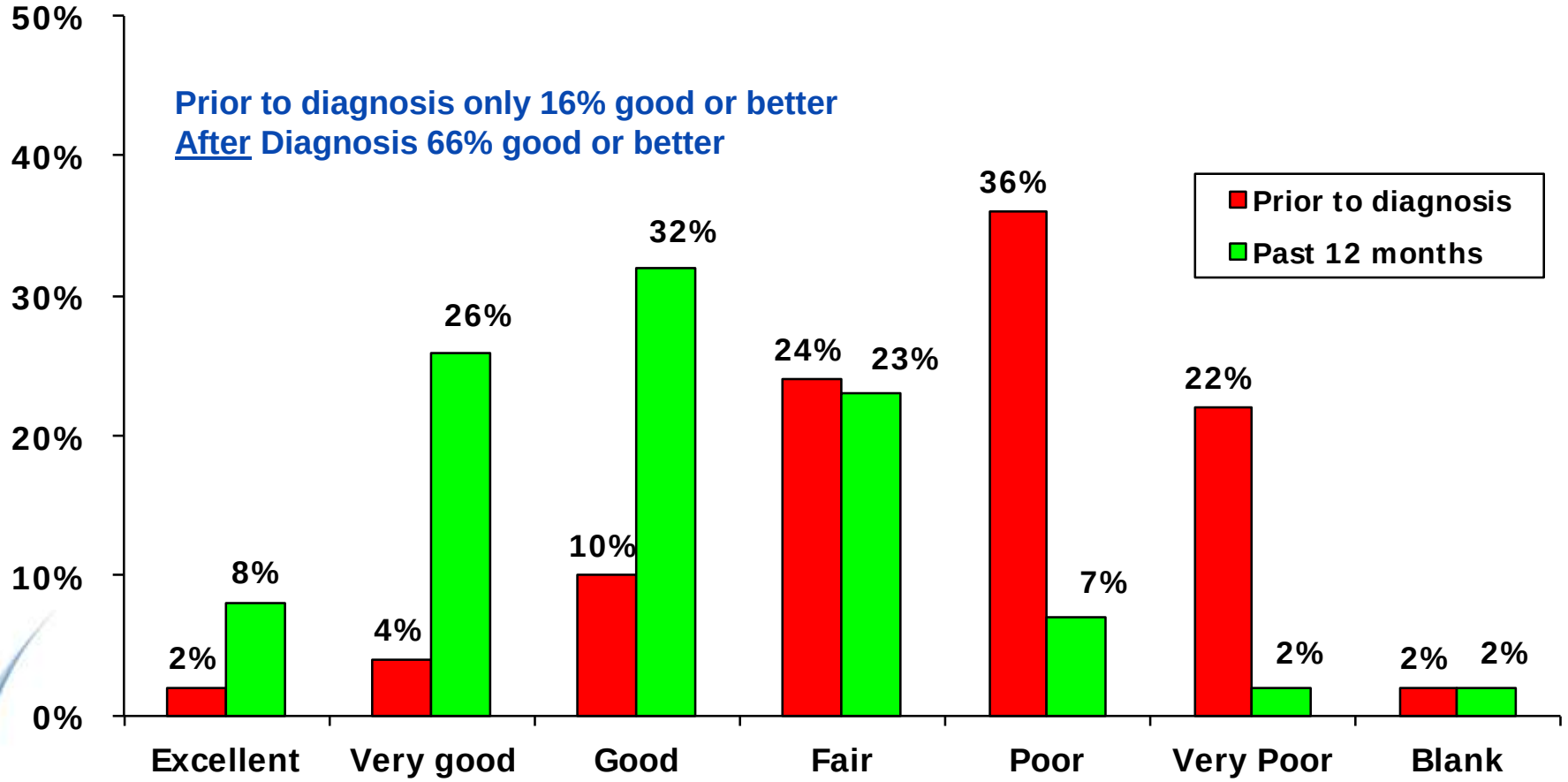


What do Patients want ?

Diagnosis

- To put a name on their condition
- To be treated for the cause of their condition rather than for the symptoms
- To get early and appropriate treatment and therefrom no/less damages

Health prior to diagnosis / past diagnosis



Q10. Would you describe his/her health in the 12 months prior to diagnosis…..? BASE: Those who are currently using IVIG or SCIG Therapy N=955

Q61. Would you describe his/her health in the past 12 months as…..? BASE: Those who are currently using IVIG or SCIG Therapy N=955

Source: 2008 IDF National Patient Treatment Survey

What do Patients want ?

Treatment

- Access to :
  - Effective, safe
  - Available, affordable therapies

IPOPI PID Patient Needs & Outlooks Survey

A Report based on 300 patient online questionnaires

Research goals and objectives

- The study has been designed to provide detail on the current landscape, outlook and needs of patients in relation to their circumstances, outlooks and treatment needs with PID
- This study explores the patient experience of PID, covering aspects from treatment and unmet needs to the impact of PID on daily and social life.
- The conjoint section asked respondents to evaluate a number of treatment options in rotation to establish unmet needs.
- Sample: N=300
  Patients & Care-givers of people with PID and treated with immunoglobulins.
  Sample sourced through national member organisations (NMOs) affiliated to the International Patient Organisation for Primary Immunodeficiencies (IPOPI).
  Sample was self-selecting amongst those invited by the NMOs.

Methodology

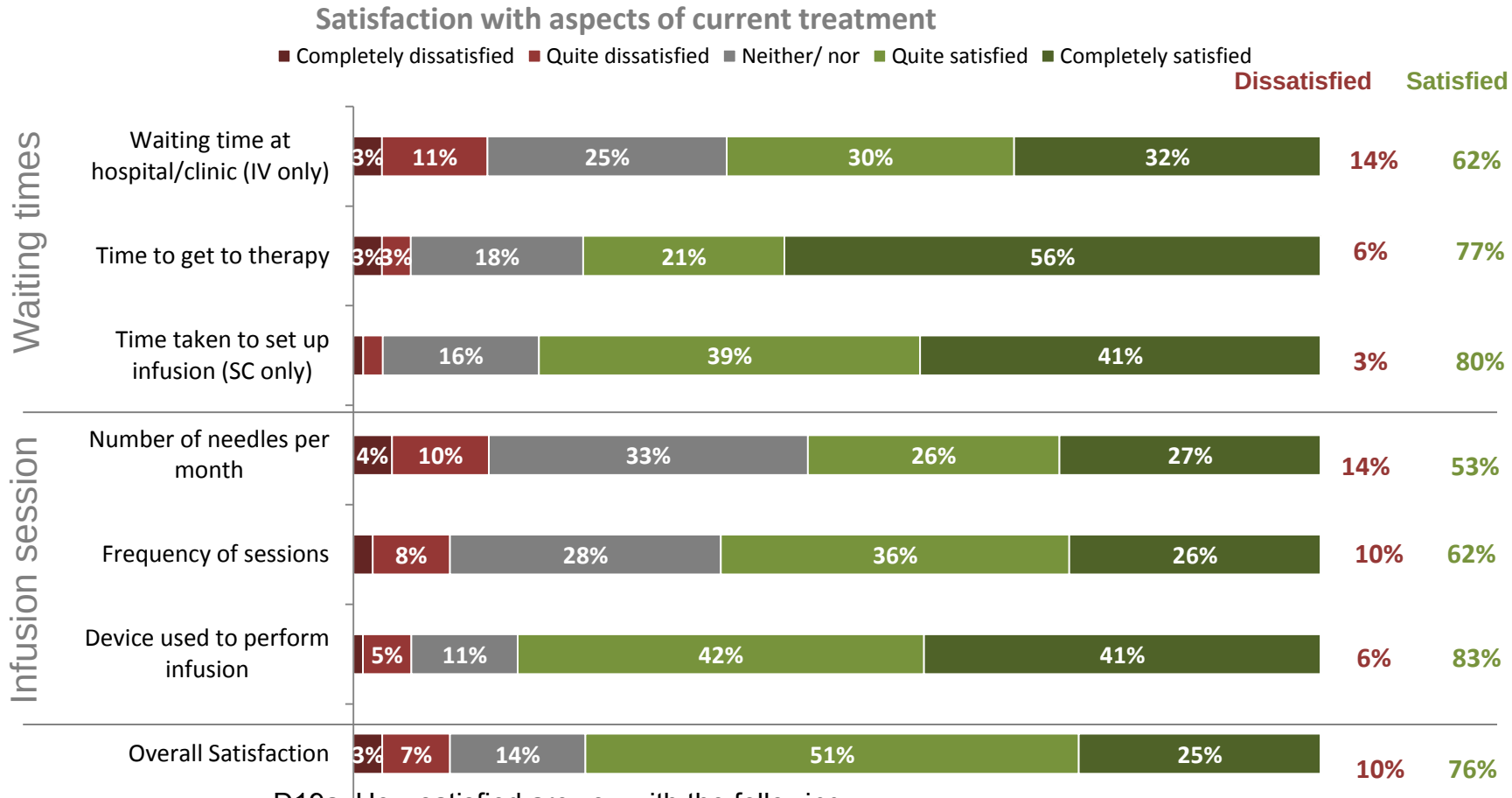
- Online questionnaire (6 languages)
- Fieldwork: 8 April – 17 October 2011

Country	Interviews
UK	59
Sweden	34
Canada	31
France	31
Germany	31
Spain	22
Portugal	21
Argentina	15
Brazil	13
South Africa	10
Colombia	9
Italy	9
Switzerland	4
Belgium	2
New Zealand	2
Poland	2
Australia	1
Austria	1
Hungary	1
India	1
Netherlands	1

>Overall, 76% are satisfied with their treatment.

>However 14% are dissatisfied with :

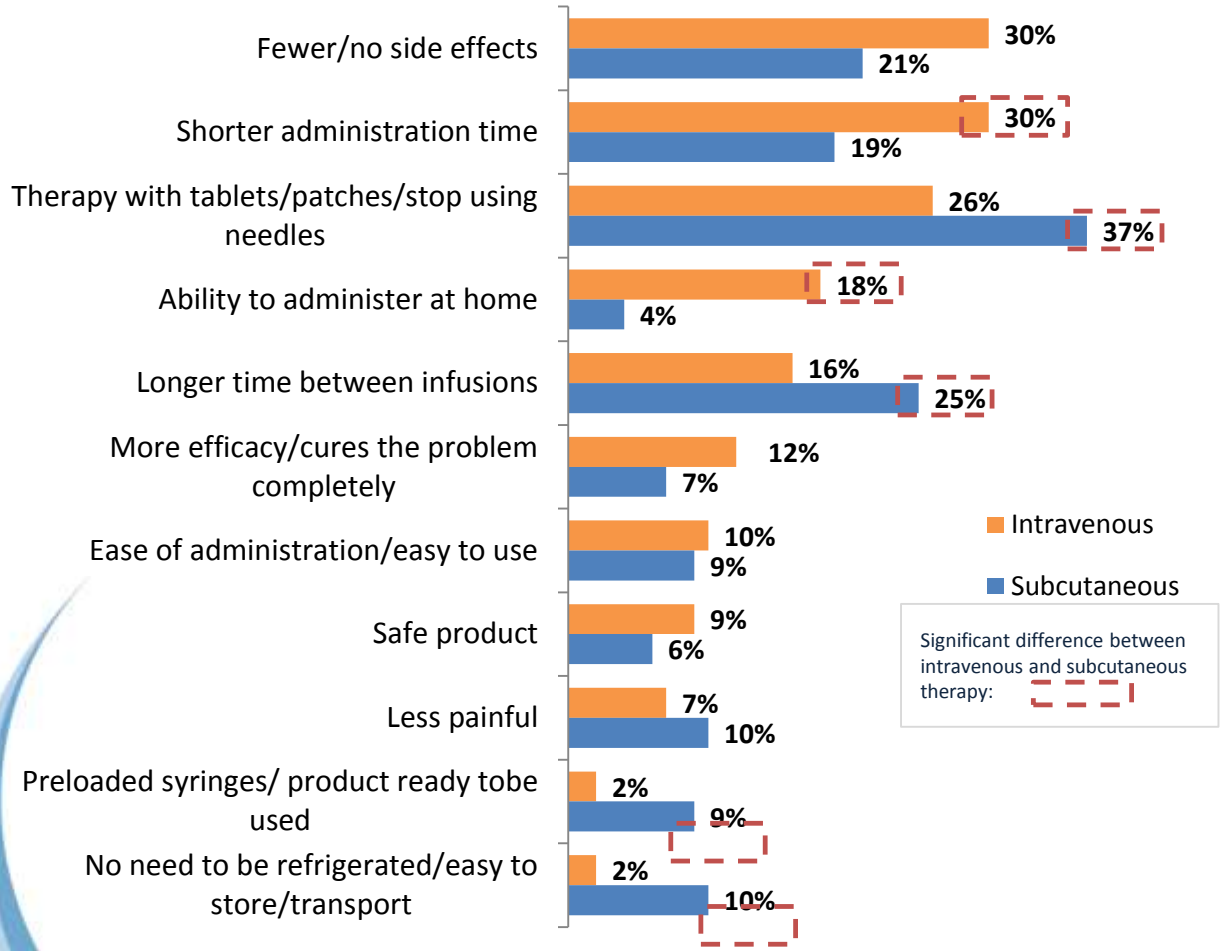
- the number of needles to contend with each month
- The waiting time at hospital or clinic



D19a: How satisfied are you with the following…
Base: All Respondents (300), SubC only (134), IV only (160)

>IV and SC patients differ regarding the features they look for in an ideal product to treat PID.

Features of an ideal treatment for PID (spontaneous mentions)

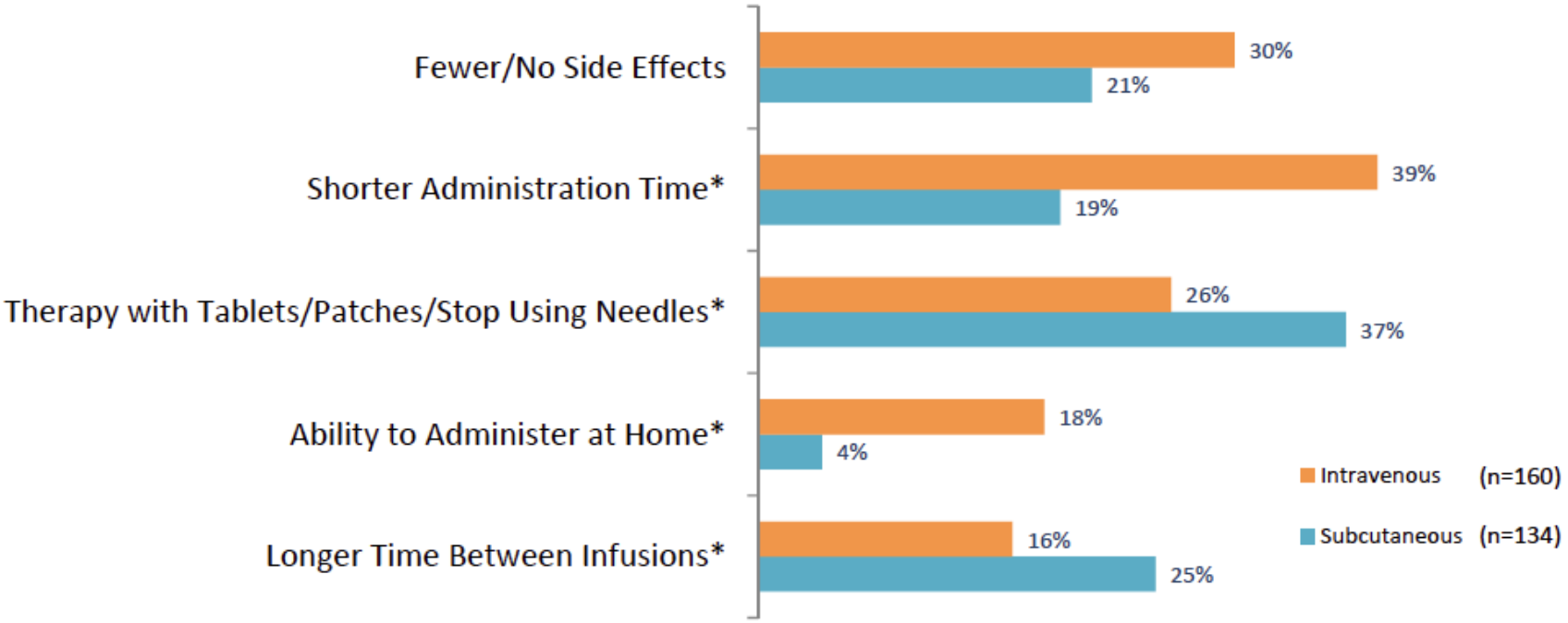


>Compared to SC patients, a significantly higher share of IV patients mentioned '**shorter administration time**' and '**ability to administer at home**' as a feature of an ideal product.

>Among SC patients '**longer time between infusions**' features more often as an attribute of an ideal product.
9% of them would like to use '**preloaded syringes**'.

D21: Thinking about future PID treatments imagine you were working with a medical design team what two features would you look for in the ideal product?
Base: All Respondents (300), Intravenous (160), Subcutaneous (134)

Spontaneous Mentions of Ideal PID Treatment Features



*Significant difference between IV and SC routes of administration

Source: 2010 IPOPI Patient Needs and Outlooks Study

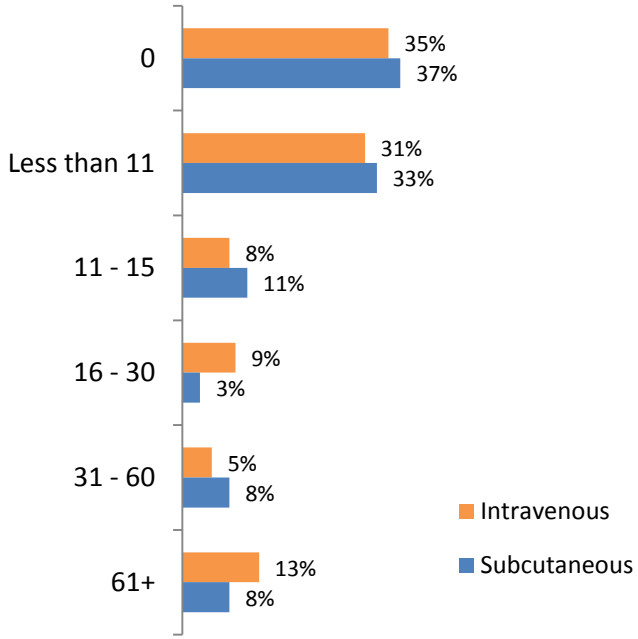
What do patients want ?

Quality of life

- Painless, simple, long lasting treatment with no-side effects
- Feel well and have a normal life

>66% (using IVIG) and 70% (using SCIG) report missing less than 11 work/school days during the past 6 months.
>Of these, 35% (using IVIG) and 37% (using SCIG) missed 0 days

Days missed at work/education due to ill health in last 6 months



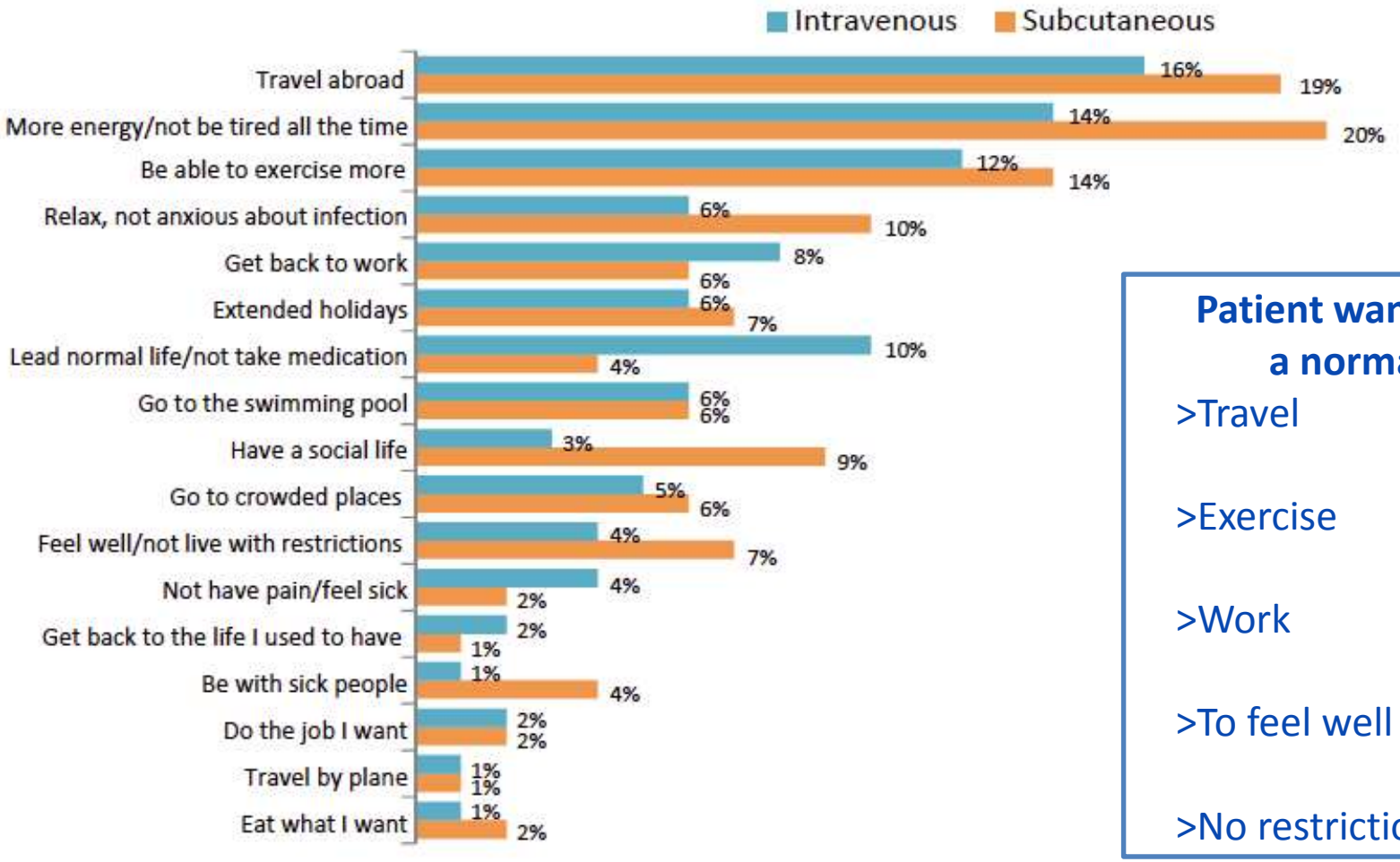
Unscheduled or emergency visits in the last 12 months

>There was no difference in the number or unscheduled visits based on the type of administration (IV vs. SC)

H4: In the last 12 months, how many unscheduled or emergency visits have you/the patient made to each of the following in relation to PID?
H5: And how many days have you/the patient missed at work/education due to ill health in the past six months?
Base: All Respondents (300)

Living with PID

« What is the one thing you would LIKE to be able to do but don't feel you can, because PID ? »



Patient want to have a normal life

>Travel

>Exercise

>Work

>To feel well

>No restrictions

Source: 2010 IPOPI Patient Needs and Outlooks Study

Barriers to Treatment from our experience in 56 countries

Medical

- Lack of knowledge/awareness by GPs.
- Very low rate of diagnosis, small number of immunologists.
- Lack of adult centres

IGs : Access and choice

- Treatment options depends on Hospital tenders.
- Hospitals providing only one IG product.
- Pharmacists considering IGs are generic
- Adults not receiving adequate treatment, trough levels below 6.
- Shortage of IVIG and SCIG.
- Need to optimize supply in developing world regions
- Access to SCIG therapy
- Access to home therapy

Barriers to Treatment
from our experience in 54 countries

Payment / reimbursement

- Still many countries with no national healthcare system reimbursement
- Children vs adults – children reimbursed, adults not: diagnosis not recognised.
- A single IG product reimbursed .
- Reimbursement based on type of PID: i.e. Children diagnosed with SCID, WAS are declared disabled => treatment is reimbursed. Less severe such as CVID are not.
- Uneven coverage with Health Insurance Companies.

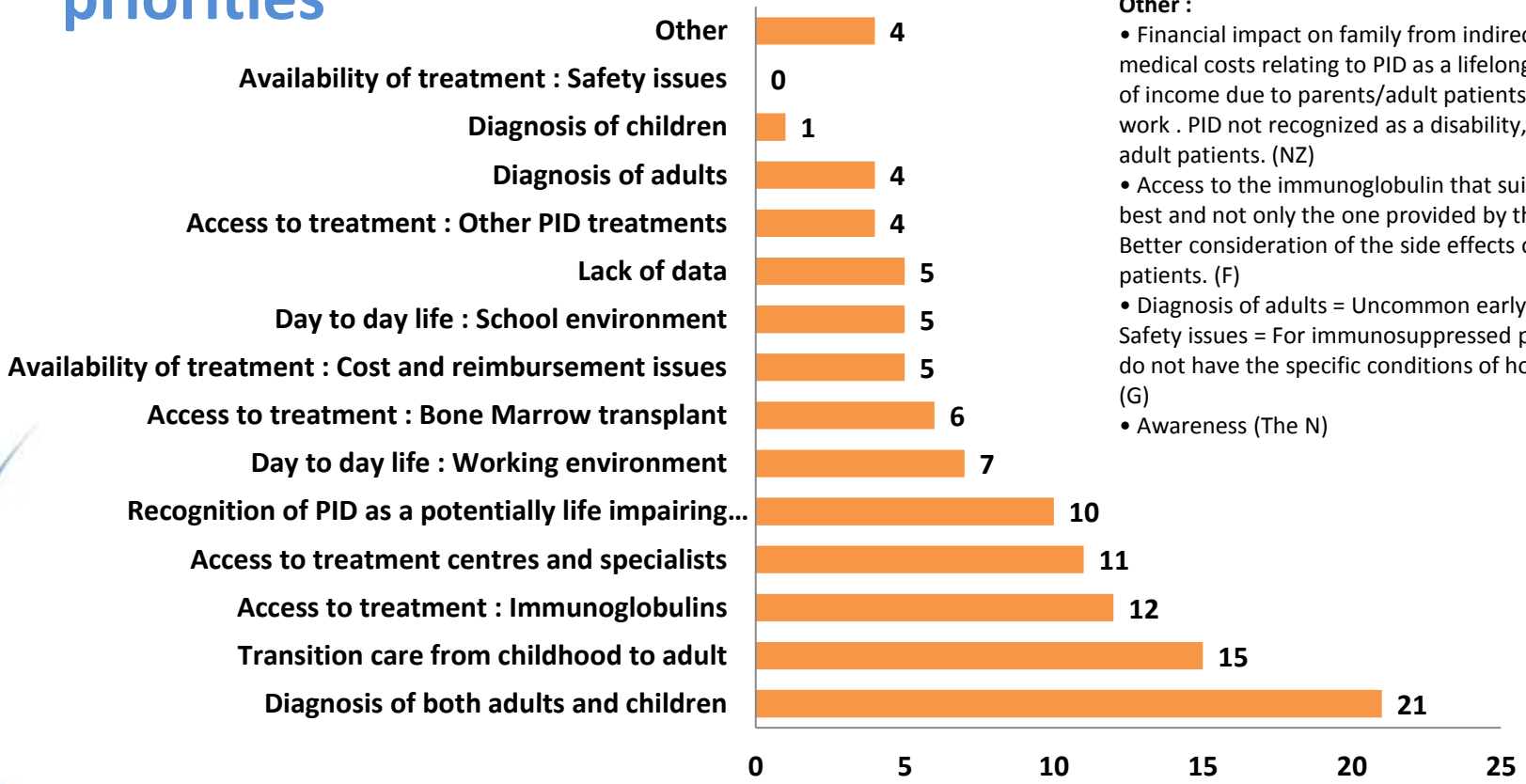
Bureaucratic processes

No prioritisation in Health Policies.

What are the 3 main areas of concern at the moment for PID Patients in your country?

IPOPI Members Survey October 2014

- **Diagnosis, transition care and access to IG top priorities**



Other :

- Financial impact on family from indirect +non medical costs relating to PID as a lifelong illness - loss of income due to parents/adult patients unable to work . PID not recognized as a disability, especially in adult patients. (NZ)
- Access to the immunoglobulin that suits the patient best and not only the one provided by the hospital. Better consideration of the side effects of the IG on patients. (F)
- Diagnosis of adults = Uncommon early diagnosis. Safety issues = For immunosuppressed patients, often do not have the specific conditions of hospitalization (G)
- Awareness (The N)

Key issues faced by IPOPI's NMOs in Asia

No IG Reimbursement
Vietnam (>6y), Indonesia Singapore

No National Registry
Indonesia, Vietnam, Singapore, Hong Kong, Thailand

No or Poor Access to SCIG
Hong Kong, Vietnam, Thailand, Hong Kong, Malaysia, Indonesia

Only one IG reimbursed
Vietnam (<6y), Thailand, Hong Kong

Under-diagnosis
All NMOs in Asia

Lack or Poor Adult Care
All NMOs in Asia

PID Principles of Care

Published 15 Dec 2014, Frontiers Immunology



frontiers in IMMUNOLOGY

HYPOTHESIS AND THEORY ARTICLE

published: 15 December 2014

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Primary immune deficiencies – principles of care

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Primary immune deficiencies (PIDs) are a growing group of over 230 different disorders caused by ineffective, absent or an increasing number of gain of function mutations in immune components, mainly cells and proteins. Once recognized, these rare disorders are treatable and in some cases curable. Otherwise untreated PIDs are often chronic, serious, or even fatal. The diagnosis of PIDs can be difficult due to lack of awareness or facilities for diagnosis, and management of PIDs is complex. This document was prepared by a worldwide multi-disciplinary team of specialists; it aims to set out comprehensive principles of care for PIDs. These include the role of specialized centers, the importance of registries, the need for multinational research, the role of patient organizations, management and treatment options, the requirement for sustained access to all treatments including immunoglobulin therapies and hematopoietic stem cell transplantation, important considerations for developing countries and suggestions for implementation. A range of healthcare policies and services have to be put into place by government agencies and healthcare providers, to ensure that PID patients worldwide have access to appropriate and sustainable medical and support services.

Keywords: primary immunodeficiencies, awareness, diagnosis, management, treatments, worldwide

INTRODUCTION

WHY A PRINCIPLES OF CARE DOCUMENT/CALL TO ACTION

Primary immune deficiencies (PIDs) are a large and growing group of over 230 different disorders, caused when some components of the immune system (mainly cells and proteins) are defective. While PIDs are generally recognized as rare disorders, some are more common than others. Taken as a whole, they represent an important group of conditions that, if not treated, can be chronic, life-long, serious, and even fatal. The lives of patients with PIDs are profoundly impacted by their condition. The immune system normally helps the body to fight infections caused by germs (or "micro-organisms") such as bacteria, viruses, fungi, and protozoa. Owing to defective immune systems, people with PIDs are more prone to infections. In addition, a poorly regulated immune system may start to attack tissues, leading to inflammation, and autoimmunity (1, 2). When PIDs are left undiagnosed or are misdiagnosed, chronic illness and disability take a heavy toll on healthcare resources (3, 4).

The immune system is divided into two parts, each of which contains two components: on the one hand, soluble proteins may be particular for one germ (antibodies) or non-specific (complement). The other components are cellular – those that are specific for one germ only (lymphocytes) and innate cells that are involved in clearing all types of infections (such as phagocytes including macrophages and neutrophils).

Primary immune deficiencies are currently classified into groups, depending on the part(s) of the immune system affected.

[†] Contributors: M. Ballow (Children's Hospital of Buffalo, USA); L. Brown (UCL Institute of Child Health, London, UK); B. T. Costa-Carvalho (Federal University of São Paulo, Brazil); J. D. Edgar (United Kingdom Primary Immunodeficiency Network, UK); W. Egner (British Society for Immunology, UK); S. Ehl (Center for Chronic Immunodeficiency, Freiburg, Germany); M. Faoor (University of Stellenbosch, South Africa); A. Garibaldi (Karolinska Institute at Huddinge University Hospital, Sweden); A. Gennery (University of Newcastle upon Tyne, Newcastle, UK); I. Kerr (Paul Ehrlich Institut, Langen, Germany); C. Klasen (UCL Institute of Child Health, London, UK); Y. L. Lau (The University of Hong Kong, Hong Kong, China); J. Litzman (St. Anna's University Hospital Brno, Czech Republic); [illegible]

PID Principles of Care lays out fundamental principles of care that should be met to reach gold standard care for PIDs.

We endorse the [….] principles, as elements of PID care provision that should be available and implemented in each country.

6 key principles

- Principle 1: The role for **specialised centres**
- Principles 2 & 3: The Importance of **Registries** and the Need for **international research collaborations**
- Principle 4 : The Role of **Patient Organisations**
- Principle 5: **Management and Treatment options for PIDs**
- Principle 6: Managing PID Diagnosis and Care **in all Countries**

IPOPI Bob LeBien Programme – S.E. Asia

First-Ever National PID Events led to launch of new NMOs:

- First ever meetings between doctors, patients and other stakeholders
- Co-organized with doctors societies
- Involvement of key PID doctors from the region & Europe
- Media coverage of events
- Political endorsement
- Calls for action
- Launch of national groups through patient-doctor collaboration in 6 countries





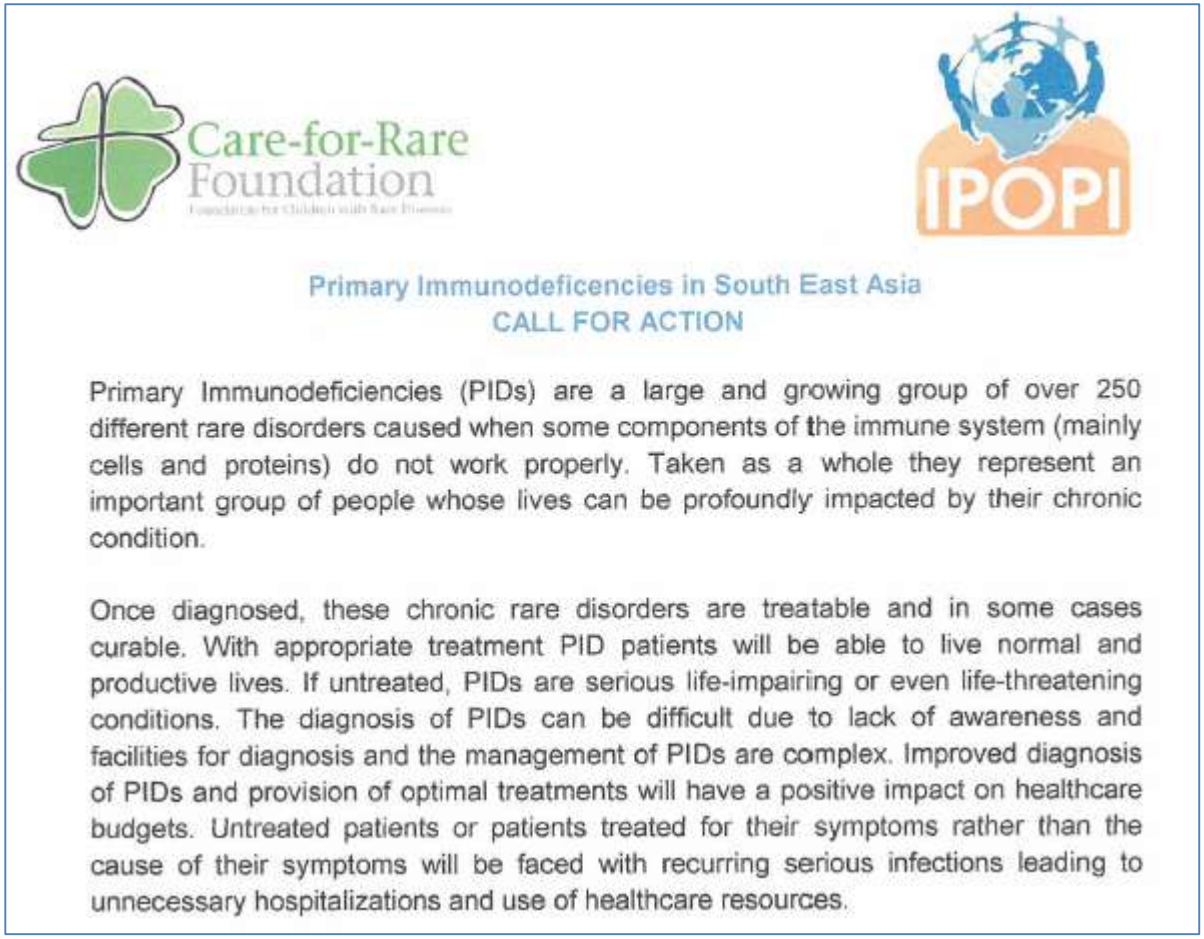
Regional Meetings and Campaigns in Asia

- IPOPI and Care-for-Rare Foundation Meeting 26th January 2015 in Bangkok
- Historical launch of regional SEA doctors group: SEAPID
- Upcoming APSID/SEAPID/IPOPI Meetings in Hong Kong, April 2016





Call for Action – PIDs in South East Asia



Care-for-Rare Foundation

IPOPI

Primary Immunodeficencies in South East Asia
CALL FOR ACTION

Primary Immunodeficiencies (PIDs) are a large and growing group of over 250 different rare disorders caused when some components of the immune system (mainly cells and proteins) do not work properly. Taken as a whole they represent an important group of people whose lives can be profoundly impacted by their chronic condition.

Once diagnosed, these chronic rare disorders are treatable and in some cases curable. With appropriate treatment PID patients will be able to live normal and productive lives. If untreated, PIDs are serious life-impairing or even life-threatening conditions. The diagnosis of PIDs can be difficult due to lack of awareness and facilities for diagnosis and the management of PIDs are complex. Improved diagnosis of PIDs and provision of optimal treatments will have a positive impact on healthcare budgets. Untreated patients or patients treated for their symptoms rather than the cause of their symptoms will be faced with recurring serious infections leading to unnecessary hospitalizations and use of healthcare resources.

- ✓ **Early diagnosis**
- ✓ **Treatment**
- ✓ **Medical Education**
- ✓ **PID registries and data collection**
- ✓ **Specialized Centres**

Next Steps – IPOPI actions

- IPOPI Regional Patients Meeting in Asia
  - Back to back with APSID Conference
  - In collaboration with SEAPID
- Launch Meetings:
  - Taiwan
  - The Philippines
- World Primary Immunodeficiency Week Events in the region
  - 22-29 April 2016

Conclusions

- Still huge **unmet need for PID treatments**
- The need for **the widest choice of available IG therapies**
- The need for **individualized therapies** and regulations that take into account **Igs are not generic medicines**
- Availability of appropriate therapies result **in effective management of healthcare budgets, active/productive patients**
- Improved patient survival in connection **to higher IG dosing** has been demonstrated
- On the other hand **growing demand** for IG therapies

Conclusions

- Importance of **stakeholder collaboration**

PLUS
PLATFORM OF PLASMA PROTEIN USERS

- Publication of **stakeholder consensus statements** since 2010
- Provide an **adequate supply of PDMPs from recovered and source plasma** to meet patient needs on a global level.
- **Non- remunerated and remunerated donations** are currently essential to meet global health needs.
- Recognise that plasma and PDMPs supply is a basic healthcare need and a safety issue. An **insufficient supply is a major safety risk to patients**.

Thank you for your attention!

