

Report and Action Plan

I. Introduction

On Friday 17 May 2013, a workshop was organised by IPOPI (International Patient Organisation for Primary Immunodeficiencies) at the Sao Paulo UNIFESP Hospital with the collaboration of ABRI (Associação Brasileira de Imunodeficiência) and ANPIC (Associação Nacional dos Portadores de Imunodeficiência Primária Congênita) and other key Brazilian medical stakeholders. Mrs Jose Drabwell, Chair of the IPOPI Board of Directors opened the meeting and after welcoming remarks invited participants to introduce themselves. Johan Prevot, IPOPI Executive Director, briefly summarized the purpose of the meeting whose main objective was to discuss and agree a joint awareness action plan for Brazil. The language of the meeting was Portuguese.

In the Workshop several presentations were made covering the following subjects:

- Overview of IPOPI tools and examples of international awareness campaigns
- Concrete examples of successful Latin American awareness campaigns
- Overview of the Brazilian Health Policy environment in terms of legislation and access to treatment
- Overview of the treatment choices in Brazil
- Overview of the patients key priorities in Brazil
- Overview of the physicians key priorities in Brazil

II. Presentations

1. Presentation of IPOPI Awareness Campaign Toolkit – Magda Lourenço, IPOPI
2. Presentation of Awareness Campaign Success Stories – Roberta Pena, IPOPI Board member
3. Brazilian Health Policy Overview & opportunities for Awareness raising campaigns Renato Strauss (FSB Communications)
4. Access to choice of IG therapies in Brazil – Beatriz Costa Carvalho, UNIFESP
5. The Brazilian PID environment: Overview of challenges and opportunities – Physicians Perspectives – Tatiana Lawrence, UNIFESP; Magda Carneiro-Sampaio, FMUSP
6. The Brazilian PID environment: Overview of challenges and opportunities – Patient and Stakeholders perspectives – ABRI perspective and ANPIC perspective and overview of activities



1. Presentation of IPOPI and its Awareness Campaign Toolkit

Magda Lourenço, IPOPI's Communications and NMO Programming Officer provided an overview of IPOPI's history and of key international awareness campaigns IPOPI has either led or participated in. Among them the World of Primary Immunodeficiencies Week campaigns launched in 2011, the reinstatement of Immunoglobulin in the World Health Organisation List of Essential Medicines and several European Parliament actions.

The different IPOPI Support Programmes and Toolkits to support National Member Organisations (NMOs) in developing their activities at national level were also presented, including IPOPI's Awareness Campaign Toolkit and IPOPI's NMO Web toolkit.

2. Presentation of Latin American Successful Awareness Campaigns



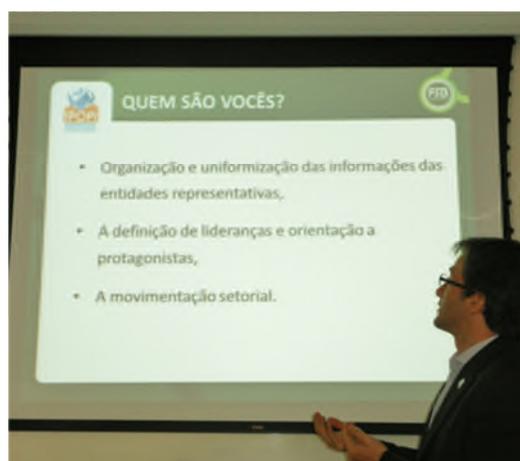
Mrs Roberta Pena, IPOPI's Board Member outlined the reasons and the ideal model for driving a successful awareness campaign and provided several concrete examples from Latin America. It was highlighted that eight of twenty Latin American countries currently have an established NMO (Argentina, Brazil, Chile, Colombia, Mexico, Peru, Uruguay and Venezuela) and three others (Costa Rica, Panama and Puerto Rico) are in the process of forming one with IPOPI's support.

Mrs Pena provided specific examples on how national and regional campaigns can be carried out successfully, stressing the example of the Call to Action signed during the 2011 LATAM High Level Summit on Primary Immunodeficiencies (back-to-back with the LASID Meeting) aiming to encourage the recognition and prioritization of PIDs at national governmental level.

3. Brazilian Health Policy Overview & opportunities for Awareness raising campaigns

Mr Renato Straus, from FSB Communications, health policy consultant for the Workshop, provided an informative overview of the Brazilian national health system organisation as well as the regulations and relevant Rare Diseases health protocols, some currently under revision.

He highlighted 2013 as a key moment to improve PID awareness in Brazil in order to engage politicians and regulators, pointing out the national rare diseases public consultation currently taking place. The importance of communications campaigns for patients' groups were emphasized as powerful tools to generate more awareness not only with the politicians but also with the general public and with physicians.



4. Access to choice of IG therapies in Brazil – Beatriz Costa Carvalho, UNIFESP

Dr Beatriz Costa Carvalho, UNIFESP, began her presentation with the LASID PID prevalence statistics per country, showing that Brazil has not yet established an accurate prevalence rate for PID.

The presentation went over the most important problems in terms of access to treatment in Brazil. PID treatment is currently made available for the patients by the Ministry of Health. However patients do not have access to their treatment of choice. Similarly, subcutaneous immunoglobulin therapies are currently unavailable. Previous advocacy efforts to change the situation have so far not been successful.

5. The Brazilian PID environment: Overview of challenges and opportunities – Physicians Perspectives



a) Dr Magda Carneiro-Sampaio, FMUSP, gave an overview of what has been done over the years in Hospital das Clínicas (part of FMUSP) which was the first PID reference centre in Brazil and provided different statistics of the number of registered patients.

It was pointed out that because Brazilians descend from a genetic mix, X-linked disorders are most commonly found and that with proper diagnosis 50 SCID babies would be expected every year.

Medical education was described as a top priority as immunology is not currently being properly taught in Brazil. Hospital das Clínicas has developed a '12 warning signs for the first year of life' leaflet currently being distributed to family doctors and paediatricians in the country.



b) Dr Beatriz Costa Carvalho and Dr Tatiana Lawrence, both from UNIFESP, provided a joint presentation with data from their latest survey of 3000 doctors to find out if some of their patients meet the PID warning signs. According to their findings most physicians are aware of PIDs (screening and treatment) but find it very difficult to properly diagnose it.

The implementation of SCID newborn screening was described as a priority in terms of access to care and diagnosis. In Brazil, newborn babies are not allowed to leave the hospital without the BCG and Polio immunization

which can cause complications and death to undiagnosed PID patients.

6. The Brazilian PID environment: Overview of challenges and opportunities – Patient and Stakeholders perspectives

ANPIC perspective and overview of activities

Marta Reis, secretary of ANPIC presented the organisation's achievements since its creation in 1996, in Minas Gerais, as a state organisation (Associação Mineira dos portadores de imunodeficiências primária congênita). Two years later they became aware of IPOPI's work and in 2007 they were registered as a National organisation, and currently represent 2506 patients.



Mrs Reis explained that throughout the 1990s' Brazilian PID patients did not have an integrated treatment plan, instead they were followed by a series of doctors focusing merely on the symptoms. Often the patients (and their families) had to take their case to court to guarantee availability of immunoglobulin supply.

ANPIC's work is still today focused on helping families and patients coping with the access to care limitations.

ABRI perspective

Dr Tatiana Lawrence, ABRI's President, presented an overview of ABRI's recent projects and events. ABRI has promoted networking between Brazilian doctors with several regional lectures and physician meetings and organisation representatives have taken part in international immunology congresses. The organisation also organises an annual patients' 'get together' meeting and have facilitated training on IVIG administration for nurses.

ABRI is establishing regional support centres (João Pessoa, Paraíba) in order to reach out to more patients and trying to support the launch of rare diseases reference centres in Brazil. Several communication materials have been produced, some of them in partnership with IPOPI's Portuguese NMO, APDIP.

ABRI has also been active at the political level and focused on getting government confirmation on the continuous supply of immunoglobulin and blood tests and on making subcutaneous Immunoglobulin available for PID patients.



III. Local strategy: agreements on potential actions and timelines

Throughout the different sessions, participants expressed their views and described their key priorities. A lively discussion ensued during which it was agreed that a joint awareness campaign should be started by both associations ABRI and ANPIC with the local support of the doctors, and with the help of IPOPI.

Participants agreed on the following **priorities**, divided in **3 categories**:

Associations:

Legal support

Collaboration between both associations

National registry of patients

Treatment:

Choice of treatment

Bone marrow transplant costs adjusted for PID (same as to oncology patients)

Subcutaneous treatment

Transition from paediatric to adult care

Diagnosis:

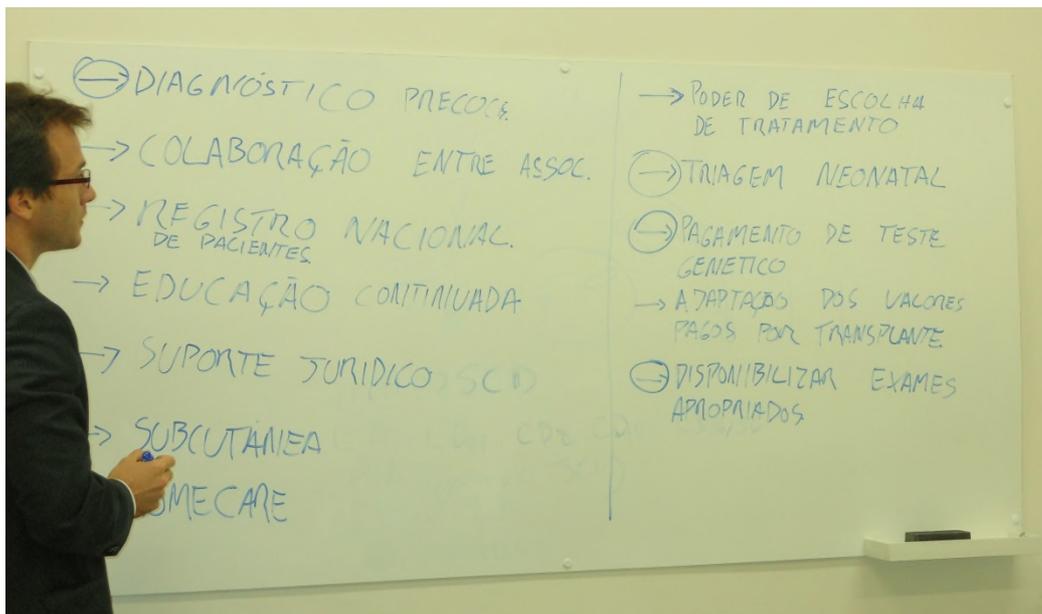
SCID Newborn screening

Genetic testing paid by National health system

Continuous medical education

Appropriate tests made available

Early diagnosis



The following list of activities and timing was identified:

| Activity | Timing | Responsibility |
|---|--------------------|--------------------------|
| <ul style="list-style-type: none"> Joint response to Ministry of Health's National Consultation on Rare Diseases carving out PIDs as an important subgroup of rare diseases deserving specific attention | 7 June 2013 | ABRI/ANPIC/IPOPI/Doctors |
| <ul style="list-style-type: none"> Letter to Ministry of Health outlining key access to diagnosis and care priorities identified during the meeting | June 2013 | ABRI/ANPIC/IPOPI/Doctors |
| <p>Political awareness and advocacy campaign targeting key Brazilian policy makers</p> | Q3 2013 Q1 2014 | ABRI/ANPIC |

| | | |
|--|------------|--------------------|
| <ul style="list-style-type: none"> • Development of key political supporters network • Increase Political visibility for PIDs in Brazil • Seek support for Joint Policy Event and Call to Action | | |
| <ul style="list-style-type: none"> • Build on political support and organize joint policy event during World Primary Immunodeficiency Week in 2014 • Launch of Brazilian PID Call to Action | Q1-Q2 2014 | ABRI/ANPIC/Doctors |

IPOPI would like to thank Baxter for their support to the IPOPI Brazilian Awareness Campaign Workshop.



IPOPI PID Awareness Campaign Workshop 17 May 2013, São Paulo, Brazil

7. List of participants

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|---------------------------|---|
| Jose Drabwell | IPOPI |
| Johan Prévot | IPOPI |
| Roberta Pena | IPOPI |
| Magda Lourenço | IPOPI |
| Dr Tatiana Lawrence | ABRI President |
| Lyranne Aquino | ABRI Member |
| Lelia Bezzan | ABRI Member |
| Luciano Pedroso Mendonça | ABRI Member |
| Lindalva Santos | ABRI Member |
| Michele Santos | ABRI Member |
| Angelica Lodovici | Ataxia-teleangectasia |
| Marta Reis | ANPIC President |
| Filadelfo Barbosa | ANPIC Member |
| Marileide Santos | ANPIC Member |
| Luciana Mendonça | ANPIC Member |
| Alexandre Fonseca e Silva | ANPIC Member |
| Dr Beatriz Carvalho | Universidade Federal de São Paulo |
| Dr Magda Carneiro Sampaio | Hospital das Clínicas da Faculdade de Medicina da USP |
| Renato Strauss | FSB Communications |
| Jeff Bakker | Baxter |
| Claudia Brabata | Baxter |
| Simone de Seixas | Baxter |

