

### I. Introduction

On Friday, 11 October 2013, a workshop was organised by IPOPI and ARPID (the Romanian Association of Patients with PIDs) in order to provide ARPID with:

- Concrete examples of successful awareness campaigns
- Overview of available IPOPI tools
- Overview of ARPID main concerns at national level
- Overview of the European and Romanian environment in terms of legislation and patient advocacy
- Establish an awareness campaign plan

### II. Presentations/Discussions

1. Presentation of the workshop objectives and participants – Jose Drabwell, IPOPI;
2. IPOPI Awareness Campaign Toolkit - Johan Prévot, IPOPI;
3. Awareness Campaign Success Stories – Johan Prévot, IPOPI;
4. Health Policy Overview & Opportunities for Awareness Raising Campaigns – Leire Solís and Cristian Luțan, Rohde Public Policy;
5. ARPID Perspective of the Romanian PID Environment, Dr. Artemiza Bâldea, ARPID;
6. Overview of recent PID activities, patient's perspective, Ana-Maria Posea, ARPID;
7. Local strategy – the way forward – agreements on potential actions and timelines - ALL

#### 1. Presentation of IPOPI Awareness Campaign Success Stories

Johan Prévot, IPOPI Executive Director, presented IPOPI, its objectives and activities, as well as the awareness campaigns success stories that have been developed at international level and in different countries. The presentation showed different examples that could be used as models by the NMOs and adapted to the specific objectives of the campaign and the audience targeted.

In this sense, Mr. Prévot gave the example of the first patient awareness campaign workshop that took place in Belgium, which set up in motion an awareness campaign endorsed by Herman

van Rompuy, the president of the European Council. He also presented examples of a campaign in Russia, where the IPOPI NMO won an important legal battle to ensure the same access to treatment throughout the country. These activities in Belgium and Russia, were based on workshops such as the one in Sibiu, and were replicated in many countries since then, most recently in Brazil, Thailand and, at a regional level, in South Africa. At international level, Mr. Prévot presented the example of the World Health Organisation list of essential medicines campaign, as well as the World Primary Immunodeficiency Week supported by IPOPI and developed by the NMOs.



Mr. Prévot also explained that actions and campaigns can be undertaken individually by NMOs, but also by working together with key stakeholders along the lines of PLUS (Plasma Users Platform– uniting organisations such as Haemophilia, Alpha-1 disorders and PIDs) or rare diseases coalitions.



## 2. Presentation of IPOPI Awareness Campaign Toolkit

Johan Prévot, IPOPI Executive Director, presented the different support programmes as well as the toolkits developed by IPOPI to support National Member Organisations (NMOs) in developing their activities at national level. Mr. Prévot presented the following tools:

|   |   |
|---|---|
| <ul style="list-style-type: none"> <li>• <a href="#">Broadcast Guidelines;</a></li> <li>• <a href="#">Facts about PID for Governments</a></li> <li>• <a href="#">Media Guidelines</a></li> <li>• <a href="#">Guidelines on Corporate Sponsorship</a></li> <li>• <a href="#">Government Outreach Guidelines</a></li> </ul> | <ul style="list-style-type: none"> <li>• <a href="#">Celebrity Guidelines</a></li> <li>• <a href="#">Family &amp; School Guidelines</a></li> <li>• <a href="#">PID Leaflet IPOPI</a></li> <li>• <a href="#">Facts about PID for the Medical Profession</a></li> </ul> |
|---|---|

## 3. Opportunities for awareness raising campaigns at political level: EU and Romanian level

Leire Solís and Cristian Luţan, Rohde Public Policy, presented an overview of the political opportunities for awareness raising campaigns. The presentation aimed at providing the participants to the workshop with an analysis of the upcoming opportunities at political level that could be used to increased awareness about PID and the situation that PID patients are facing in Romania. One of the main opportunities identified was the commitment by the Romanian Minister of Health, Mr. Eugen Nicolăescu, to develop a national plan for rare diseases by the end of 2013. This would be a very good occasion for ARPID to get involved into the drafting process and increase its activities for awareness-raising of PIDs at a national level.

## 4. ARPID Perspective of the Romanian PID Environment



Dr. Artemiza Bâldea, President of ARPID and a PID patient, presented the activities of ARPID to date, and the challenges that PID patients face in Romania.

Thus she explained that:

- There are currently 350 patients diagnosed in Romania, of which 35 adults and 68 children are undergoing treatment;
- It is very likely that the number of actual PID patients is higher, as many doctors are not aware of PIDs;

- Access to treatment for new patients remains a problem as it depends on the size of the available public budget, although improvements have happened in the last years;
- Treatment is available in one-size fits all, as public auctions to procure it, only allow one type of product per year to be acquired;
- Support to patients is currently lacking in several regions of Romania, mainly in Bucovina, Moldova, Muntenia and Dobrogea;

## 5. Overview of recent PID activities, patient's perspective

Ana-Maria Posea, vice-president of ARPID, presented her perspective as a PID patient and explained she had to wait for 9 years before a diagnosis. She highlighted that the challenge of getting a confirmed diagnosis for many patients still remains. Without the confirmed diagnosis, patients cannot be included on the treatment list. It would be important, she added, that the existing national rare diseases programme be taken up by more centres in the country. This would increase the number of healthcare professionals that are aware of PIDs.

## 6. Local strategy – the way forward: agreements on potential actions and timelines

Throughout the different sessions, participants expressed their views on what could be ARPID strategic objectives and the support needed from IPOPI. The participants agreed on the following items:

### Treatment

- Getting the right amount and the right dosage for immunoglobulin (IG);
- Increase coverage for more PIDs, as some, such as congenital neutropenia, or hereditary angioneurotic edema (HAE), or anti-fungal prophylaxis are not covered;
- Subcutaneous treatment (SCIg) which is not available;

### Diagnosis

- PIDs are underdiagnosed;
- There is a long delay between diagnosis and actual treatment access;
- There is no effective newborn screening
- Limited coverage of investigational tests
- There are discrepancies between adult and children care
- Large distances between diagnosis centres

### Structural

- Need for a specialists centres' network for adults (a paediatric network already exists);
- Lack of qualified/ recommended centres
- Need for a national registry of PID patients



The participants agreed on the following objectives:

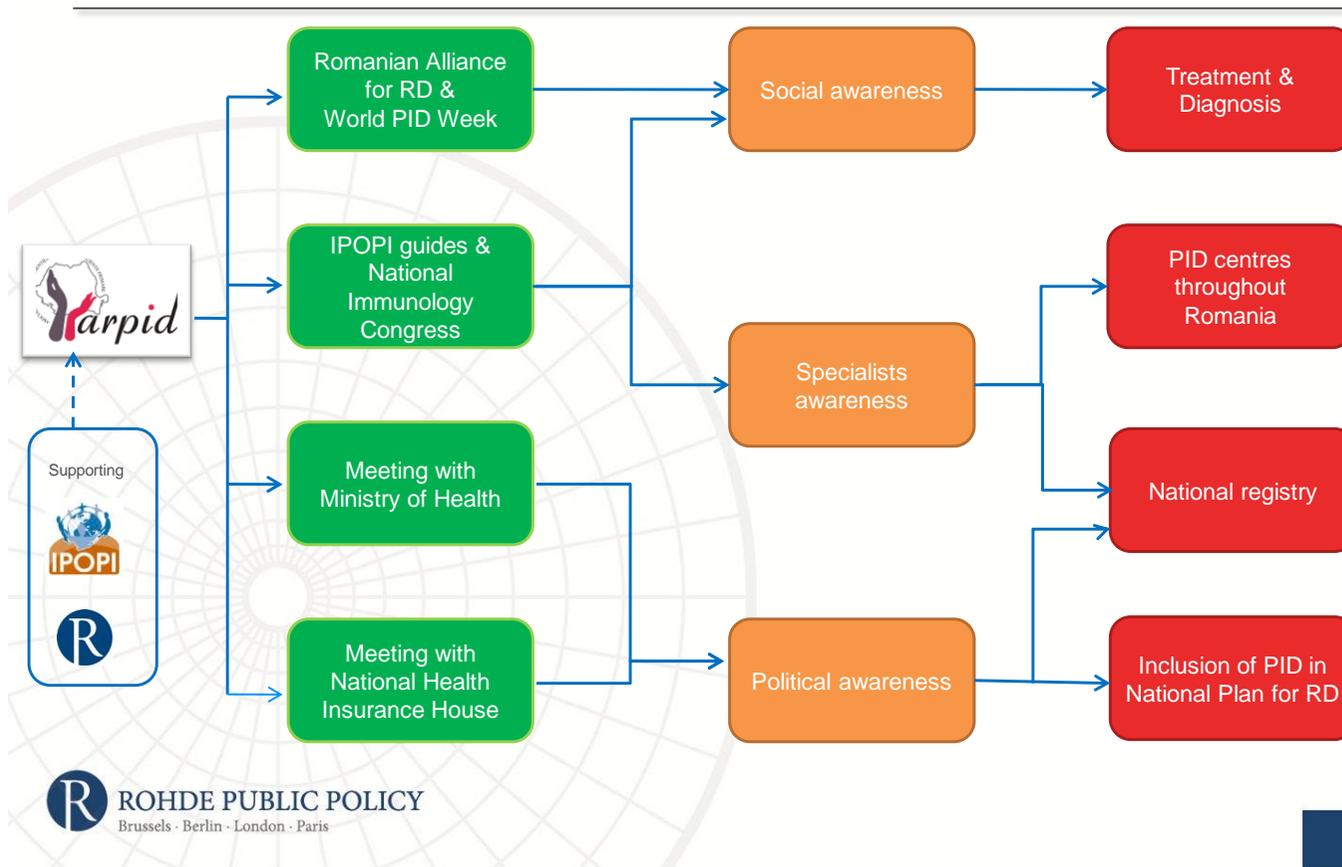
| <b>Direct/Practical patient care issues</b>  | <b>Structural care issues</b>   |
|--|---|
| <ul style="list-style-type: none"><li>• Improve PIDs knowledge of healthcare professionals (GPs and specialists)</li><li>• Extend ARPID patients' support to more regions (e.g. Moldova)</li></ul> | <ul style="list-style-type: none"><li>• Ensure the voice of PID patients is heard in the development of the National Rare Diseases Plan</li><li>• Extend the network of PID coordinators under the national rare diseases programme</li><li>• Create a national registry for PIDs (ESID format)</li></ul> |

The participants agreed that for achieving the above-mentioned objectives, it is important to focus on the envisaged National Plan for Rare Diseases.

The participants agreed on the following list of activities and timing:

| Activity   | Timing  | Responsibility   |
|--|---|--|
| <p><b>Development of the national rare diseases plan</b></p> <ul style="list-style-type: none"> <li>• Contact national rare diseases programmes coordinators at the Ministry of Health and National Health Insurance House</li> <li>• Drafting of meeting request and brief to support PID awareness raising meetings</li> <li>• Affiliate to Romanian Alliance for Rare Diseases</li> </ul>   | <p>November 2013</p> <p>October 2013</p> <p>Q4 2013</p>                       | <p>ARPID</p> <p>IPOPI with support from RPP</p> <p>ARPID</p>   |
| <p><b>Awareness raising campaign among medical community:</b></p> <ul style="list-style-type: none"> <li>• Draft a letter to local health insurance houses to support the recruitment of PID coordinators</li> <li>• Presentation at National Immunology Congress on PIDs to improve awareness among specialists</li> <li>• Translate and publicise the IPOPI-EU expert guidelines</li> <li>• Organize a meeting of different PID centres</li> </ul> | <p>Q4 2013 – Q1 2014</p> <p>Q4 2013</p> <p>Q1-Q2 2014</p> <p>Q1 – Q2 2014</p> | <p>ARPID</p> <p>ARPID</p> <p>ARPID with support from IPOPI</p> <p>ARPID with support from Octapharma</p> |
| <p><b>Awareness raising campaign among society:</b></p> <ul style="list-style-type: none"> <li>• Participation in the World PID Week</li> </ul>  | <p>Q1– April 2014</p>   | <p>ARPID with support from IPOPI</p>   |
| <p><b>Political awareness campaign:</b></p> <ul style="list-style-type: none"> <li>• Develop contacts with local MPs from Timis, Cluj counties</li> </ul>  | <p>Q4 2013 – Q1 2013</p>  | <p>ARPID</p>   |

Tools & Supporters → Targets → Objectives



### III. List of participants

|                        |  |
|------------------------|--|
| Jose Drabwell          | IPOPI  |
| Johan Prévot           | IPOPI  |
| Artemiza Bâldea        | ARPID President                                |
| Ana-Maria Posea        | ARPID Vice-President                           |
| Stefania Neacșu        | ARPID Secretary                                |
| Florian Blaga          | ARPID Member                                   |
| Mihaela Degetaru       | ARPID Member                                   |
| Nicoleta Giugiuc       | ARPID Member                                   |
| Dzenita Subasic        | Bosnia-Herzegovina patient group observer      |
| Dr Mihaela Bataneant   | Victor Babes University of Medicine, Timisoara |
| Prof Dr Victor Cristea | Iuliu Hateganu University of Medicine, Cluj    |
| Dr Miron Nicolae       | Iuliu Hateganu University of Medicine, Cluj    |
| Dr Iurian Sorin        | Paediatrician, Sibiu                           |
| Leire Solis            | Rhode Public Policy                            |
| Cristian Lutan         | Rhode Public Policy                            |
| Livia Baltag           | Octapharma                                     |

