

## **Growing older with a PID: transition of care and ageing**

### **Recommendations of the International Patient Organisation for Primary Immunodeficiencies (IPOPI)**

**July 2017**

#### **Introduction**

The International Patient Organisation for Primary Immunodeficiencies (IPOPI) is the association of national patient organisations, dedicated to improving awareness, access to early diagnosis and optimal treatments for people with primary immunodeficiencies (PIDs) worldwide.

PIDs are a group of more than 300 genetic rare disorders in which the immune system does not work adequately or at all. PIDs are often regarded as a group of conditions typically observed in paediatric age. However, PIDs affect patients throughout their life, and with increasingly more knowledge and awareness about rare diseases, more attention should be paid to specific stages in life where support is key in ensuring the well-being and the quality of care of the patient, such as during adolescence, early adulthood and at later stages in life. Moreover, improved medical care, scientific developments and increased knowledge about PIDs have led to greater survival in paediatric patients, so that the cohort of patients entering the adult age is now increasing, with all the repercussions this may entail in terms of services and support needed.

The IPOPI Recommendations on transition of care and ageing with a PID are aimed at providing an overview of the key issues faced by PID patients when entering adulthood and at a later stage in their life, as well as supporting patient representatives in their advocacy efforts, healthcare professionals in their practice and encouraging policy-makers to implement a patient-centred approach in the decision-making.

The Recommendations are based on the outcomes of the IPOPI 9<sup>th</sup> PID Forum on “Growing older with a PID: transition of care and ageing”, chaired by the Member of the European Parliament Dr Elena Gentile (S&D, Italy) and supported by Mr Carlos Zorrinho (S&D, Portugal) in June 2017 at the European Parliament (Brussels, Belgium).

## I. Transition of care for PID patients

In most European countries, as teenagers with PIDs enter adulthood, they are moved to adult immunology services. This change requires special arrangements to help young people, in order to ensure the continuation of safe and quality PID care: this is known as transition of care.

Transition of care is usually defined as a *“purposeful, planned process that addresses the medical, psychosocial and educational/vocational needs of adolescents and young adults with chronic physical and medical conditions as they move from child centred to adult oriented health care systems”*<sup>1</sup>. To this, it should also be added the daily organisational issues and needs of patients. For PID patients, this process is often overlooked, but it is a cost-effective way of ensuring compliance with treatment and preventing possible adverse effects. To this end, it is vital to develop a patient pathway<sup>2</sup> of transition of care and an integrated transition of care in specialised centres while also ensuring associated cost coverage of the treatment the patient will need throughout his/her adult life.

### Developing a pathway of transition of care

Transition pathways need to take into account the multiple aspects of the maturing process of the young adult along with social and personal changes that will affect the diagnosis, treatment, treatment compliance, and autonomy of the future adult with PID<sup>3</sup>.

1. Policy-makers and patient advocates should work towards a coordinated patient-centred transitional care pathway guiding young patients to adult services to subsequently increase patients' compliance with the treatment and reduce unnecessary costs associated with health damage.
2. Young patients, supported by their families, should learn whether the PID centre they regularly attend offer special help for young people. This can range from running out-of-hours clinics to help fitting appointments around the young patient's schedule, special clinics for young people, self-care clinics or “parent-free” consultations.

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<sup>1</sup> Department of Health, Transition: getting it right for young people, available at: [http://webarchive.nationalarchives.gov.uk/20130123205838/http://www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsPolicyAndGuidance/DH\\_4132145](http://webarchive.nationalarchives.gov.uk/20130123205838/http://www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsPolicyAndGuidance/DH_4132145), consulted on 28 June 2017.

<sup>2</sup> Patient pathway is defined as the “route that a patient will take from their first contact with a national health service member of staff (usually their GP), through referral, to the completion of their treatment.” Available at: [http://webarchive.nationalarchives.gov.uk/+http://www.dh.gov.uk/en/Healthcare/Primarycare/Treatmentcentres/DH\\_4097263](http://webarchive.nationalarchives.gov.uk/+http://www.dh.gov.uk/en/Healthcare/Primarycare/Treatmentcentres/DH_4097263), consulted on 28 June 2017.

<sup>3</sup> N. Mahlaoui, K. Warnatz, Al. Jones, S. Workman, A. Cant, Advances in the Care of Primary Immunodeficiencies from Birth to Adulthood, *J Clin Immunol* (2017) 37:452-460. DOI 10.1007/s10875-017-0401-y.

## Integrating transition of care

Transition from paediatric to adult age is already difficult for a healthy person. For PID patients, it is more complicated. Changing physicians, caregivers and hospitals can be very difficult for a young person. Young patients and parents often develop particularly strong relationships with their paediatric care team. Consequently, transferring to adult care could be discouraging. Thus, the process needs to be thoughtfully planned ahead.

Great Ormond Street Hospital (GOSH) and the Royal Free London NHS Foundation Trust in London or Necker-Enfants Malades University Hospital in Paris are a great example of collaboration developed for PIDs transition through their established best practice guidelines and integrated care pathways for all adolescents with PID requiring transition.<sup>4</sup>

3. Healthcare professionals together with the patient and his/her family should plan a transition from paediatric to adult PID clinic in advance. Patient needs should be jointly identified and assessed by the patient and the specialised healthcare professional on an individual basis maintaining the most optimal continuity of care possible.
4. Specialised treatment centres for paediatric and adult PID care should work closely together to guarantee patients and their families a smooth transition from paediatric to adult care. Paediatric and adult doctors, nurses, physiotherapists, social workers and patients should continuously share their knowledge and information on the patient's specific PID, contributing to a better management of the transition.
5. Transition of a young person with PID from the paediatric unit to an adult centre should also include patient empowerment by developing the young person's autonomy, empowerment and self-reliance through special care services for young patients and providing the patients clear and comprehensive information about treatment and care options.

## Ensuring associated costs coverage

Patients with PIDs should have access to the treatments they need in a continued and timely fashion. Governments and healthcare authorities should ensure that the treatments are available and affordable for these patients. Coverage of transition of care by the health insurance is a critical element in ensuring smooth transition for the patients. Depending on the country's health system, people with PIDs can be covered by their national health insurance or require a health insurance plan.

6. Young people with PIDs, with the support of their families or doctors, should check whether care for adults with PIDs in an adult clinic is covered by their health

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<sup>4</sup> Ibid. and [www.la-suite-necker.aphp.fr](http://www.la-suite-necker.aphp.fr)

insurance (either the one provided by the national health system or by a private health insurance).

7. Health insurance coverage should be in place for young adults with PIDs, when moving from the paediatric centre to adult health services and from then on throughout their lives.

## II. Ageing with a PID

Growing older with a PID can be especially challenging for patients and requires a complete adaptation of young and adult patients to their environments, leading to a better physical, psychological and social impacts on their lives. These aspects are prerequisites for an autonomous and active lifestyle. When a person reaches a late stage in life, often he/she will need to deal with conditions associated with ageing, such as diabetes, coronary heart disease or hypertension just to name a few.

With developments in medical science in general, but also in the field of immunology, where clinical management has evolved tremendously with better diagnosis and more treatment options in a broad perspective for all PID patients, an increased number of adults with PIDs are reaching older age.

The European Society for Immunodeficiencies (ESID) Registry shows that in 2013, there were 774 patients registered with a PID in Europe over the age of 65, which represents 5.45% of the total patients registered<sup>5</sup>. Most of these patients suffer from common variable deficiency (63.29%), followed by isolated IgG subclass deficiency (11.71%) and other agammaglobulinemias (7.08%)<sup>6</sup>.

In spite of the increasing numbers of people with a PID reaching old age, there is still little research that has been done on the different aspects of what it means to be an elderly PID patient. To this end, and as stated by the European Commission, socio-economic research in the field of rare disease care provision/organisation should be supported both at Member States level and at European level.<sup>7</sup>

### Promoting research about PIDs in elderly persons

Much of the information available to treating physicians is based on extrapolations either from other patient cohorts' results (i.e. from patients suffering from cystic fibrosis to understand the benefit of antibiotic prophylaxis) or from younger patients.

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<sup>5</sup> Verma N et al. Drugs Aging. (2013) Therapeutic Management of Primary Immunodeficiency in Older Patients. DOI: 10.1007/s40266-013-0079-7

<sup>6</sup> Ibid.

<sup>7</sup> Commission Expert Group on Rare Diseases, Recommendations to support the incorporation of rare diseases into social services and policies, available at: [https://ec.europa.eu/health/sites/health/files/rare\\_diseases/docs/recommendations\\_socialservices\\_policies\\_en.pdf](https://ec.europa.eu/health/sites/health/files/rare_diseases/docs/recommendations_socialservices_policies_en.pdf), consulted on 28 June 2017.

8. Clinical guidelines should be developed to ensure that care provided to persons with PIDs is adequate in this population.
9. More research should be done on the benefits of standard therapies for PIDs in elderly population and the potential interaction of such therapies with the most common co-morbidities in this stage of life.

### Meeting patient-specific needs

Elderly patients living with a PID and suffering from co-morbidities associated with age require even more specific attention. Close consideration needs to be made on whether PID patients suffering with diabetes are receiving immunoglobulin replacement therapy with low levels of glucose containing carbohydrates, for instance, or whether those patients who have a low manual dexterity can receive their immunoglobulin replacement therapy intravenously.

10. Elderly people with PIDs have specific needs that require an individualised approach, based on the clinical history, pathologies linked to their specific PID, dexterity of movement, other conditions they may suffer from and any potential long-term treatment side-effects they may experience.
11. Elderly PID patients often receive informal care from family members, which is often unpaid and provide the non-stop support, especially in the case of the most debilitating forms of PIDs. For this model to be sustainable, Member States should acknowledge this type of care and develop mechanisms to identify and support informal caregivers.<sup>8</sup>
12. Ageing PID patients still in their working age but unable to work due to their condition, should be provided adequate support by ensuring financial independence through smooth bureaucracy and communication between the health care and social security systems.

Home treatment is an important aspect of primary immunodeficiency care, especially in the elderly community, therefore:

13. Member States, through national rare disease plans, should introduce a framework to ensure accessible and sustainable home care and long-term care services for elderly rare disease patients, including PID patients.

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<sup>8</sup> World Health Organisation, *Active Ageing: Good health adds life to years*, available at: [http://www.euro.who.int/\\_\\_data/assets/pdf\\_file/0006/161637/WHD-Policies-and-Priority-Interventions-for-Healthy-Ageing.pdf?ua=1](http://www.euro.who.int/__data/assets/pdf_file/0006/161637/WHD-Policies-and-Priority-Interventions-for-Healthy-Ageing.pdf?ua=1), consulted on 23 June 2017

## Promoting healthy ageing

Health promotion is a cost-effective way to prevent further illness development in PID patients resulting in a longer hospital stays, bacterial infections and increased risk of cancer.

14. Healthy ageing, should be addressed through a comprehensive strategy with actions on individual, organisational and societal levels. Targeted measures should be taken with regards to infrastructure, the supply of adequate health care and care services, the provision of education and information for all PID patients, as well as their participation in the economic, political and social life.

## Implementing ICT solutions

Information and communication technologies (ICT) can help improve patients' health, address systemic challenges for healthcare systems and support the transition from a hospital-based health care model to a patient-centred and integrated one, while respecting the digital privacy and data protection of patients.

15. eHealth solutions (e.g. mobile apps, electronic health records etc.) should be used as cost-effective tools to support the transition from a hospital-based healthcare to a patient-centred and integrated one, improve access to affordable care, and contribute to the sustainability and resilience of healthcare systems.
16. Digital education for patients, should be promoted and provided to all patients regardless of their age or condition.

As of September 2017, the recommendations are endorsed by:

**Members of the European Parliament**

**Dr. Elena Gentile (S&D, Italy)**

**Carlos Zorrinho (S&D, Portugal)**

**Sirpa Pietikäinen (EPP, Finland)**

**Isabella De Monte (S&D, Italy)**

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**Patient Representatives**



### Other signatories

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