

OCTOBER 25

IPOPI WHIM WORKSHOP

Meeting report



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IPOPI recently had the pleasure of bringing together key opinion leaders in the field of WHIM syndrome to an online workshop, focused on understanding the current state of data collection on WHIM throughout the world and identifying the unmet needs remaining. The aim of this workshop was to discuss the value that an international WHIM Syndrome Data Harmonisation Platform could bring for the health care professionals caring for WHIM patients around the world, and for the patients suffering from this very rare condition. This workshop is the next step in IPOPI's disease-specific activities for WHIM syndrome, following the recent WHIM leaflet and Clinical Care Webinar.

The rationale behind this workshop was to investigate the need for an innovative artificial intelligence-based global platform that would embed patient self-reported data to potentially follow the natural history of the disease longitudinally, retrospectively, and systematically. This would allow for a better understanding of global and regional trends in WHIMS diagnosis, progression, and treatment. The project could, importantly, also seek to gain a better understanding of treatments used for WHIMS, clinical outcomes, impact on quality of life, the burden of treatment and overall impact on healthcare resource utilisation.

During presentations on “WHIM data collection around the world” and “patient experience and expectations” the following points were raised:

- All WHIM stakeholders play an important role in understanding the natural course of the disease. Increasing and coordinating the collection of data on WHIM can improve our knowledge and understanding of this disease, as well as its co-morbidities.
- All WHIM experts presenting on smaller registries, databases or cohorts highlighted that WHIM patients often are diagnosed late and/or misdiagnosed and/or lack a proper diagnosis altogether. Diagnosis delay is a common problem, as well as potential underdiagnosis. It was indicated that the data is siloed and that better harmonisation would bring benefits.
- The data presented by the experts indicated a variety of different symptoms and early signs in the different small registry studies. This was understood as the characteristics of small numbers, further highlighting the need for expanding and harmonising the data collection.
- Patients with WHIM and their families still feel isolated and lack information about how WHIM syndrome may impact their lives. This is improving, but more work is needed.
- There is a need to develop common knowledge and language to learn about the experience of WHIM patients. Additionally, it is essential that patients and patients’ families understand the methodology and the language, in turn creating better involvement in research projects and registries.
- Committing patients to research helps to translate research into daily life for patients.

Following this first informative session, a presentation on how an artificial intelligence-based data collection platform could work was provided, allowing for the attendees to raise their questions regarding how to execute such a project. This was followed by an open discussion with participants highlighting the unmet needs on data collection they have identified in their practice, as well as the potential they see for this project.

KEY TAKEAWAYS AND NEXT STEPS

There are many disparities around the world regarding how data are collected. Participants were positive towards international collaboration and agreed that harmonising efforts on data collection for WHIM could ensure a better understanding of treatments and how it can change the natural course and burden of the disease.

Patients who are actively involved in research can substantially enhance the data collection. A data harmonisation platform could open the possibility of more patient-reported outcomes.

The objectives of the platform need to be carefully designed to avoid over-complicating the project, as well as comply with it being an evolving project.

There is a further need to understand the priorities of both health care professionals and patients.

Recommendations for treatment of WHIM syndrome still vary, and further research is needed to evaluate the long term evolution of risks and benefits of treatments.

Doubts were raised regarding the costs of data collection which remains an issue despite a potential harmonisation platform. It was agreed that this is far from trivial, but that the establishment of a registry could still be beneficial.

It was agreed to organise a follow-up meeting to further identify needs and refine the project, including the consideration of a governance structure.

CONCLUSION

The aim of this WHIM workshop was to bring together both health care professionals and patient representatives to discuss a potential data harmonisation platform for WHIM syndrome. IPOPI was very pleased with the outcomes of this meeting and look forward to continuing working on this project and further refining the details of this important initiative in the months to come, starting with the upcoming follow-up meeting.

Participation list

Shariq Ali, Vice President, Medical Affairs, X4 Pharmaceuticals

Raffaele Badolato, University of Brescia, Italy

Diego Cadavid, Head medical advisor X4 Pharmaceuticals

David Dale, Division of General Internal Medicine, University of Washington, USA

Femida Gwadry-Sridhar, CEO & Founder Pulse Infoframe

Natalie Helena, IPOPI Events Manager

Pamela Lee, The University of Hong Kong

Nina Liu, Director, Clinical Product Management, Pulse Infoframe

Nizar Mahlaoui, Chair IPOPI Medical Advisory Panel, Past Chair of ESID-registry, CEREDIH, France

Philip Murphy, National Institute of Allergy and Infectious Diseases, National Institutes of Health, MD, USA

Peter Newburger, UMASS Memorial Health and Boston Children's Hospital Boston, MA, USA

Julia Nordin, IPOPI NMO Programmes Manager

Martine Pergent, IPOPI President

Johan Prevot, IPOPI Executive Director

Michele Rhee, Vice President, Patients Affairs and Advocacy, X4 Pharmaceuticals

John Seymour, Chair Immune Deficiency Foundation, IPOPI Board member, USA

Anna Shcherbina, Rogachev Federal Scientific and Clinical Centre of Pediatric Hematology, Oncology and Immunology, Moscow, Russia

Kylle Tollefsen, Director, Medical Communications and Publications, X4 Pharmaceuticals

Sharon Vacher, WHIM patient representative expert, Australia

Jolan Walter, University of South Florida at Johns Hopkins All Children's Hospital, USA

Leanne Wicks, WHIM patient representative expert, Australia

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