

Raising Awareness and Shaping Policy on Plasma-Derived Medicinal Products: A Patient Advocacy Case Study

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INTRODUCTION

Plasma-derived medicinal products (PDMPs) are essential, life-saving therapies for people living with rare, chronic, and life-threatening conditions, including primary and secondary immunodeficiencies, bleeding disorders, and autoimmune diseases. Despite their recognition as critical medicines at a European level, access to PDMPs remains fragile, with recurrent supply disruptions, regulatory challenges and inequalities across Member States.

OBJECTIVE

This poster presents the Plasma Protein Users Working Group (PUPP) in the Czech Republic as a patient-led advocacy case study focused on raising awareness of the importance of plasma-derived therapies and strengthening the patient voice in discussions on sustainable availability and access.

MATERIALS AND METHODS

Established in 2024 under Rare Diseases Czech Republic (ČAVO), PUPP brings together patient representatives across multiple diagnoses using plasma-derived therapies. In 2025, the group implemented a coordinated advocacy and awareness strategy combining the launch of:

- a dedicated Czech-language information platform on plasma proteins (www.plazmaticke-proteiny.cz),
- publication of patient stories, expert interviews, and educational content,
- development of a patient position paper on PDMPs availability,
- structured engagement with policymakers, regulators, healthcare professionals, and journalists at national and international levels.

Figure 1. Number of patients using other or critically important PDMPs in Czech Republic from 2012 to 2023

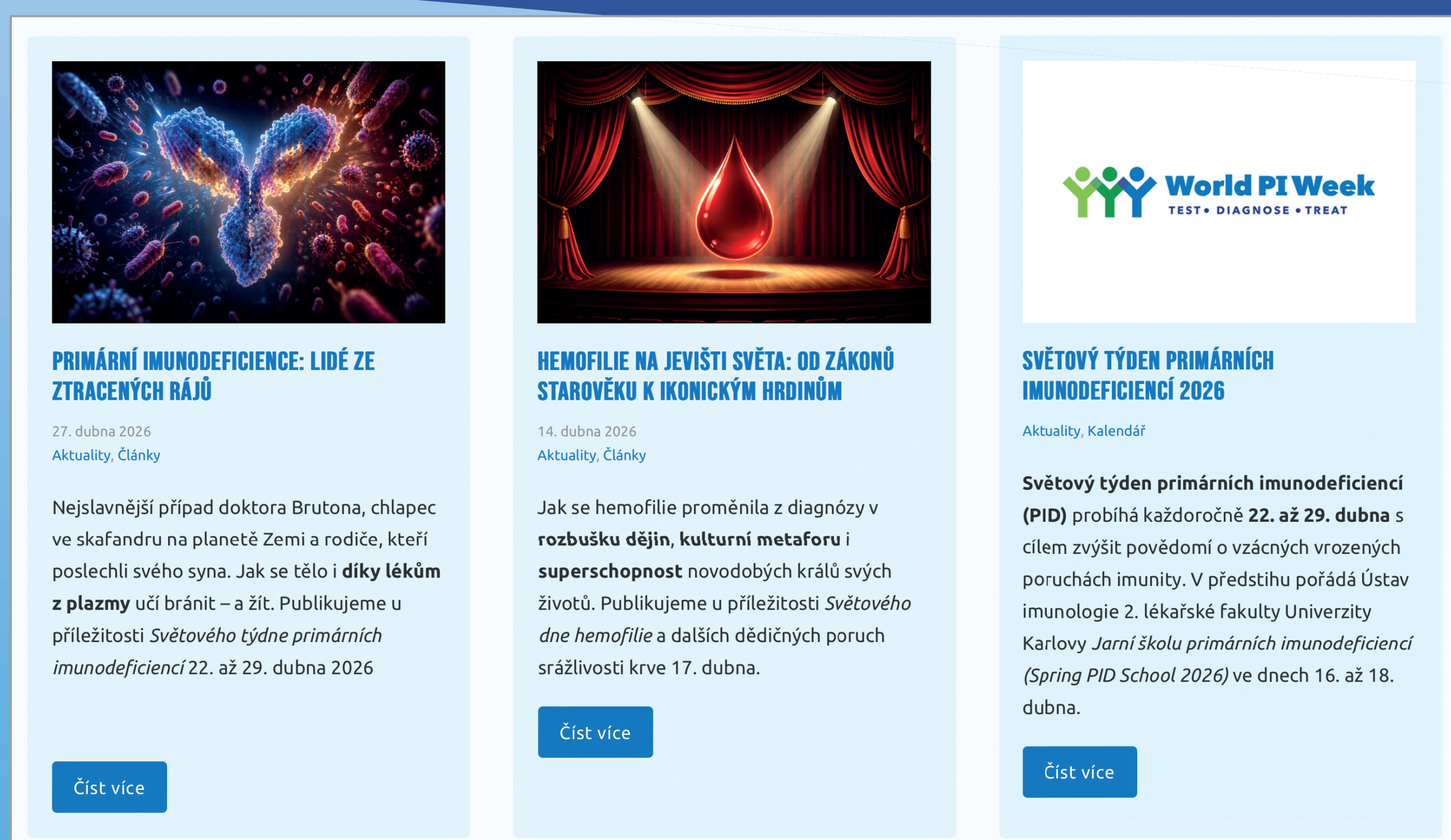
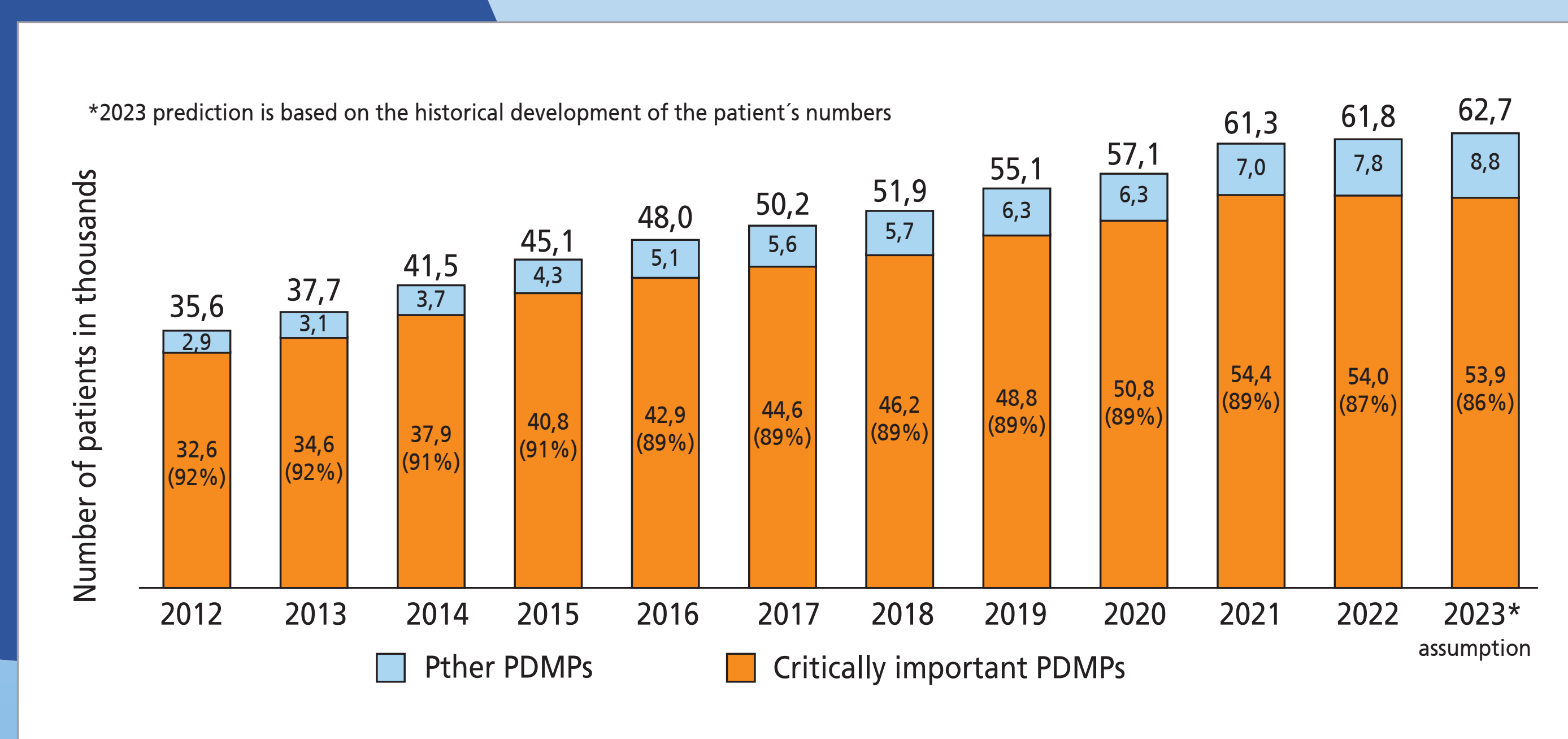


Figure 2. Dedicated awareness website available at www.plazmaticke-proteiny.cz (Czech language only)

Position Paper of the Patient Working Group on Plasma Protein Users (PUPP) – Key Points

1. **Continuous availability:** Plasma-derived medicines are critical for 62,000+ Czech patients (88% with life-threatening diagnoses); EMA classifies many as „critically important medicines.“
2. **Supply shortages:** In 2023, 10 of 19 active substances in this category faced supply gaps in the Czech Republic, with immunoglobulins and albumins hit hardest.
3. **Low immunoglobulin use:** Czech consumption (52 kg/million inhabitants) is well below the EU average (81 kg/million), reflecting unmet medical needs.
4. **Prescribing rights:** Recommend expanding prescribing authority to a broader range of physicians, especially where secondary immunodeficiency (SID) arises as a complication of other conditions.
5. **Patient-centered reimbursement:** Advocate for reimbursement rules that support subcutaneous (home-administered) therapies where clinically appropriate, factoring in patient preference.
6. **Ethical plasma donation:** Support donation campaigns while opposing aggressive or emotionally manipulative marketing and patient stories that misrepresent current medical practice.
7. **Legislative changes:** Call for patient interests to be central in upcoming EU legislative changes (Critical Medicines Act, SOHO regulation, critical medicines list).

Figure 3. English-language summary of patient advocacy group position paper

RESULTS

- This patient advocacy resulted in the first patient-driven national research study on usage of plasma-derived therapies in the Czech Republic.
- Awareness-raising activities increased visibility of patient needs related to PDMPs availability
- The patient working group position paper provided evidence-informed patient input on patient priorities and preferences.
- As a result, the working group enabled coordinated patient representation in policy dialogue, media engagement, and international scientific forums.

CONCLUSIONS

This case study illustrates how a structured, cross-diagnosis patient working group can successfully combine awareness-raising with evidence-informed advocacy to shape discussions on access to plasma-derived medicinal products. By uniting patient experience, expert input, and policy engagement, the PUPP model strengthens the patient voice in a highly complex therapeutic area. Importantly, this approach is transferable to other countries and disease areas where patients rely on critical medicines and face similar challenges related to availability, affordability, and sustainability.