# 2023/2024 Activity Report for VHL Europa

**Introduction**

The Board of VHL Europa is pleased to share the activity report for 2023/2024, sketching the achievements, challenges, and ongoing efforts of our organisation. VHL Europa serves as the umbrella organisation for 10 national VHL groups across Europe, representing the interests of approximately 20,800 VHL patients in Europe. Together, we strive to address the unique needs of our community, fostering collaboration and advocacy to improve patient outcomes and quality of life.

**Key Activities in 2023/2024**

1. **Advocacy and Engagement**
    - **Belzutifan Access Challenges:**
      Throughout 2023/2024, VHL Europa continued its advocacy efforts regarding access to belzutifan in the EU, a promising treatment for VHL patients. Belzutifan is the first systemic drug for VHL and was already approved by the FDA in the US in 2021. In October 2023 MSD (Merck Sharp & Dohme) applied for marketing authorisation in the EU. We work closely with MSD towards the implementation of compassionate use programmes. Many VHL patients in Europe are frustrated in addition to clinical staff. Advocacy around this issue remains a key focus.
    - **Meetings with the European Medicines Agency (EMA):**
      VHL Europa was invited to participate in discussions with the EMA twice this year, representing the voice of VHL patients across Europe.
    - Meeting with EURORDIS

      VHL Europa had several contactpoints with EURORDIS, the European umbrella organisation for alliances for rare diseases, to ask for support regarding the compassionate use programme.
2. **Monthly Coordination Meetings**
   Regular monthly virtual meetings via Teams allowed board members to stay updated, share resources with member organisations, and coordinate advocacy efforts. This collaborative approach ensured alignment on pressing issues, particularly regarding treatment access.
3. **Young Adults Initiative**
   Recognising the unique challenges faced by young adults with VHL, a dedicated meeting for this group was held in Munich, Germany, in May 2024. This initiative aimed to provide tailored support and resources to address their specific needs. We thank the organisers for their extraordinary efforts to make this important initiative happen. Former participants are now taking responsibility in their national alliances, i.e. as working as volunteers or working in their boards.
4. **Educational Contributions**
    - **European Renal Association Congress:**
      VHL Europa was invited as a guest speaker at the European Renal Association Congress in Stockholm, showcasing our expertise and efforts in rare disease advocacy.

5. **New Website Development**
   With funding support of €6,000 arranged by VHL Netherlands (Belangenvereniging VHL), VHL Europa developed a new website to enhance accessibility and information sharing. After selecting a provider and finalising the design, the website will go live in late-November 2024. Contributions of text and photographs from member organisations are welcomed to further enrich this resource.

6. **First Publication**
   VHL Europa successfully released its first scientific publication, marking a milestone in sharing patient-driven data and insights to influence care practices across Europe. See: https://pubmed.ncbi.nlm.nih.gov/39327187/

**Challenges in 2023**

- **Access to Treatment:**
  Despite belzutifan being under consideration at the EMA since October 2023, the lack of access remains a significant source of frustration for 100% of EU VHL patients and clinical staff. Approximately 80% of patient inquiries received by VHL Europa were related to belzutifan.

- **Diverse Health Systems:**
  Operating across 27 European countries with different health systems and 24 official EU languages continues to pose challenges in harmonising advocacy efforts and resource distribution.

**Looking Ahead**

The challenges and achievements of 2023 set the stage for our ongoing work in 2024. Key priorities include:

- Continuing advocacy for equitable access to belzutifan and other treatments.
- Expanding resources for young adult VHL patients.
- Launching and promoting the new website as a central hub for information and support.
- Strengthening relationships with EMA and other regulatory bodies to advocate for VHL patient

**Acknowledgements**

The Board of VHL Europa extends our gratitude to all members, national organisations, and partners for their dedication and contributions. Together, we are driving meaningful change for the VHL community.

Sincerely,

Board, VHL Europa