Supporting rare disease patient advocacy groups in drug development and Health Technology Assessment

A Realise Advocacy Pilot Report

realise advocacy

Rare disease patient group involvement in drug development and HTA is essential but not all are able to engage effectively to aid decision making

- **Patient involvement in drug development and access is essential** especially in rare diseases
- **Not all PAGs are able to engage effectively** in these processes. Challenges include:
    - Not having a drug development engagement strategy or planning ahead for access
    - Limited expertise
    - Insufficient resources
    - Lack of time
- **Rare disease PAGs are struggling to obtain the resources they need to engage** in the drug development and HTA processes or to identify the right kind of **practical support** to address these challenges.

- **Pharma companies have very different approaches to how they support and engage with PAGs** and what's considered appropriate support.
- **It can be difficult for pharma to engage meaningfully with PAGs** who have limited time, resources and access plans

SUCCESS FACTORS

Having a strategy

Engaging early

Staff resources

Skills and expertise

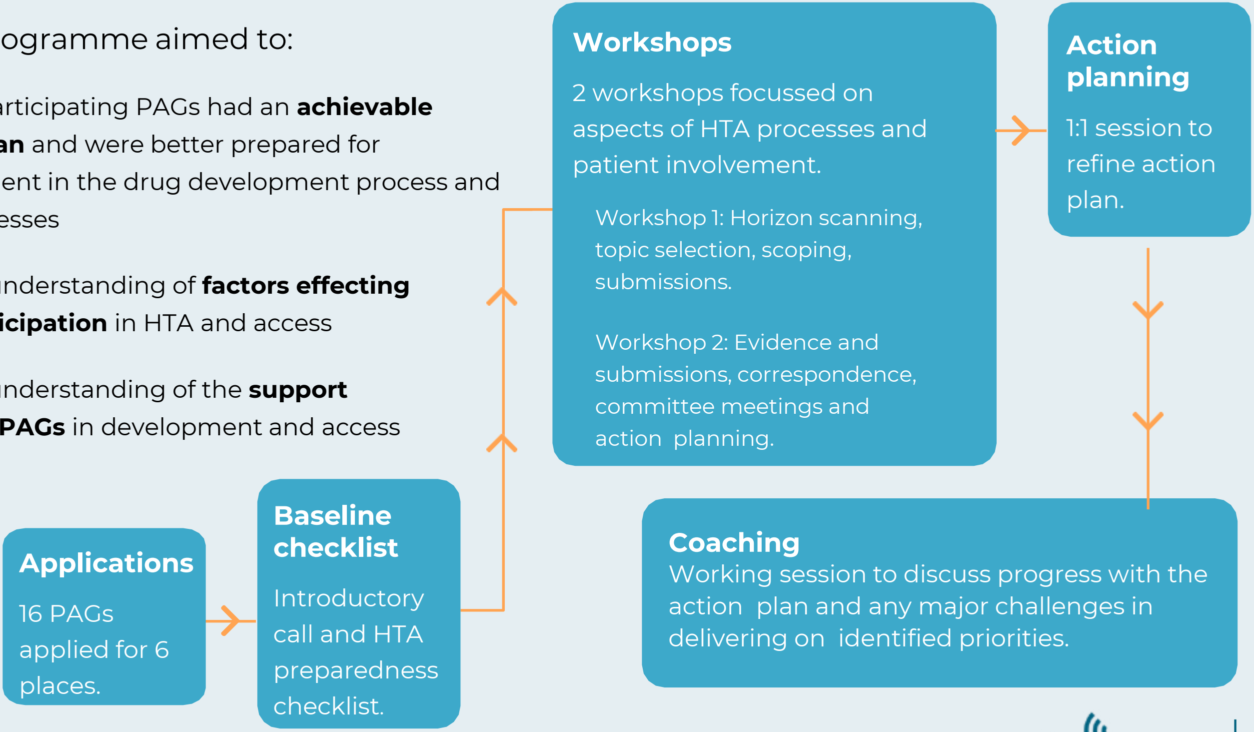
Funding available

Time

Realise Advocacy undertook a pilot programme to better understand rare disease PAGs' needs and test an approach to providing practical support

Our pilot programme aimed to:

- Ensure participating PAGs had an **achievable action plan** and were better prepared for engagement in the drug development process and HTA processes
- Test our understanding of **factors effecting PAG participation** in HTA and access
- Test our understanding of the **support needs of PAGs** in development and access



Applications

16 PAGs applied for 6 places.

Baseline checklist

Introductory call and HTA preparedness checklist.

Workshops

2 workshops focussed on aspects of HTA processes and patient involvement.

Workshop 1: Horizon scanning, topic selection, scoping, submissions.

Workshop 2: Evidence and submissions, correspondence, committee meetings and action planning.

Action planning

1:1 session to refine action plan.

Coaching

Working session to discuss progress with the action plan and any major challenges in delivering on identified priorities.

There is a gap between rare disease PAGs' needs and the available resources and support

- **Participating PAGs identified a need for practical advice and support** to effectively engage in drug development and access processes
- **Companies agreed that this was a real need for rare disease PAGs**
- **Companies had a range of views** about how this support should be resourced including that support:
  - Could be supported by individual companies with restrictions on their involvement and the selection of PAGs
  - Should be provided from a centralised fund
  - Should be provided pro bono
  - Should be provided by a charity (though it could impact on other financial support for the that charity)

> "We have vital knowledge and experience to share, but we are not necessarily equipped to share it in the most constructive and useful manner to inform HTA"

> "We lack the time of someone who has the expertise. We can develop the expertise, but we also need to off-load some of the other things to other people so the charity keeps running."

- 16 rare disease PAGs applied for support:
  - **4** had some knowledge of the HTA process
  - **14** had less than 5 members of staff/volunteers.
  - **2** had dedicated access staff/volunteers
  - **0** had prepared or planned access as part of their overall strategy
  - **3** PAGs had not engaged in earlier stages of drug development
  - **All 6 PAGs** who participated in the pilot were struggling to obtain funds to support work in this area

Rare disease PAGs reported that the structured support programme had improved their understanding and confidence to engage in HTA

Increased confidence and a **better understanding** of the requirements of HTA and **how to prioritise activities**.

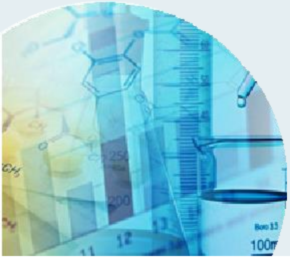
Feeling better able to **translate their experiences into evidence** and a desire to strengthen data on their patient community. All groups **identified a need to enhance their data** to support this.

Identified additional **individual support needs** such as evidence generation on current burden of illness.

Valuing training **delivered by people with experience** from HTA/payer, patient advocacy and industry.

Valuing sharing their experiences and **learning from peers**.

Increased **understanding of how to identify financial resources** and other support, but ongoing uncertainty as to whether they would actually access this support.



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Equitable and sustainable access to practical support from the early stages of drug development through to HTA would enable all rare disease PAGs to engage effectively

"Realise Advocacy took us through the process one stage at a time, and Ican honestly say that without their support, we would not have been able to interact with the review as effectively as we did."

- **More practical support for rare disease PAGs needs to be available.** This should address:
    - **Planning ahead** to ensure they can engage with every stage of drug development and access (strategic planning support)
    - **Funding applications** and ensuring resources are in place
    - **Evidence generation**
    - **Present evidence** for maximum impact
    - **Working with stakeholders** including industry
    - **Coaching and advice** at each stage to support implementation
- **A centralised fund could ensure sustainable and equitable access** to support for all rare disease PAGs and ensure all PAGs are able to engage effectively in drug development and access processes
- **Greater clarity from the ABPI, NICE** and other key stakeholders about acceptable support for PAGs could enable more funding in this area.
- Some companies engage well with rare disease PAGs but others would benefit **from support to understand the unique needs of smaller PAGs when engaging with industry.**

Realise Advocacy

We work with all stakeholders to **support patient advocates to maximise their impact in drug development and Health Technology Assessment** and transform their real-world experience into evidence.

We work with PAGs to:

- **Understand their unique circumstances**, strengths and challenges
- **Identify** their knowledge, evidence, resources, gaps and priorities
- **Build pragmatic action plans**
- **Provide practical support and coaching** to maximise impact

We work with industry and other stakeholders to ensure they can engage effectively with all PAGs and to support their patient involvement in drug development and access processes.

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Acknowledgments

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Realise Advocacy is working to build equitable and sustainable support for rare disease PAGs involvement in drug development and access

Realise Advocacy will support patient involvement in drug development and access through:

1. **Providing practical support to individual rare disease PAGs** involved in drug development and HTA
2. Working with partners to drive a **sustainable and equitable approach** to supporting all rare disease PAGs
3. **Supporting companies to maximise the impact of their engagement** with rare disease PAGs

We provide practical support to individual PAGs

1. 6-month support programme that will include:
   - Understanding PAGs’ current skills, knowledge and capacity
   - Agreeing individual SMART goals and action plan
   - Targeted support including videos, webinars, toolkits and templates. Topics could include
     - planning for drug development and access
     - generating and presenting evidence
     - funding applications
     - working with industry
   - 1:1 coaching to provide practical advice and support for planning and implementation
   - Funding for the 6-month support programme would be c.£5,000 per PAG.

2. Additional tailored support for PAGs to support delivery of action plans.

We work with partners to drive a sustainable and equitable approach to supporting rare disease PAGs

- **Promote the 6-month support programme** to hard-to-reach rare disease PAGs and develop a governance process to ensure ABPI compliance and the independence of advice and support

- **Meet with NICE** about the importance of practical support for PAGs and how funding can be appropriately secured

- **Identify supportive companies and patient organisations** and explore a joint approach to developing sustainable and equitable support for rare disease PAGs

- **Drive discussions with ABPI, BIA, EMIG** and others to establish broad support for a practical approach to supporting PAGs in drug development and access. Activities could include:
    - Develop short position paper with partner organisations
    - Discussion at BIA RDIG group
    - (Virtual) roundtable with key partners to explore sustainable and equitable solutions

- **Present at key rare disease events** to raise awareness of the specific needs of rare disease PAGs and how they can be supported

- Funding requirements would vary according to activities. Sponsorship of c.£10,000 would enable the production of a short position paper and/or a roundtable event.

We work with companies to maximise the impact of their engagement with rare disease PAGs

Companies are increasingly engaging early and often with rare disease PAGs. However, they are not always able to ensure this engagement is as effective as possible:

- **Some rare disease PAGs struggle to engage** with pharma companies due to limited knowledge, skills, time and confidence.
- **Some companies take a "one size fits all" approach to patient engagement that does not reflect the unique challenges of individual patient organisations** and may have concerns about what is appropriate when supporting PAGs.

Realise Advocacy can support company level patient engagement through:

- **Providing independent advice and support to PAGs** to help them to work effectively with industry.
- **Facilitate company training and events** with patient organisations, bringing experience and understanding of the unique needs of rare disease PAGs.
- **Review company engagement plans, initiatives and literature** to ensure they maximise the chances of successful engagement with rare disease PAGs:
  - Identify shared priorities
  - Ensure initiatives and literature are well understood by PAGs
  - Advise on approach to projects and events to maximise PAGs likelihood of effectively engaging

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