

# Supporting rare disease patient advocacy groups – case studies

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# Case Study 1: Navigating NICE and NHS England

<b>Who</b>	<b>Support</b>
<p>Small ultra rare disease PAG Small paid staff, run by father of two children with the condition Marketing Authorisation granted to a repurposed drug. No sponsor for NICE submission</p>	<p><b>Strategic guidance</b></p> <ul style="list-style-type: none"><li>• Reviewed correspondence from NICE, NHS England and the brand name manufacturer</li><li>• Identified key challenges and proposed solutions for PAG to present</li></ul> <p><b>Correspondence</b></p> <ul style="list-style-type: none"><li>• Helped prepare correspondence with NICE and NHS England highlighting key issues and solutions</li></ul> <p><b>Meeting preparations</b></p> <ul style="list-style-type: none"><li>• Briefed full team ahead of all stakeholder meetings to advise on the key issues and how to present them for maximum impact</li></ul>
<b>Need</b>	<b>Impact</b>
<ul style="list-style-type: none"><li>• Navigating NICE and NHS England processes for a repurposed drug that had been granted marketing Authorisation but, due to the arrival of generics, had no sponsor for the NICE process.</li></ul>	<ul style="list-style-type: none"><li>• Successfully removed treatment from NICE appraisal route and achieved commissioning via NHS England</li></ul>

# Case Study 2: Preparing for committee meetings



Who	Support
<p>Small ultra rare disease patient organisations</p> <ul style="list-style-type: none"><li>• Two small PAGs (1-3 staff) in same disease area</li><li>• No licensed treatments</li><li>• NICE HST process underway</li></ul>	<p><b>1:1 coaching</b></p> <ul style="list-style-type: none"><li>• The role of the committee meeting in the overall HTA process</li><li>• What to expect from the committee meeting</li><li>• Identifying key points</li><li>• How to engage and present for maximum impact</li></ul>
Need	Impact
<ul style="list-style-type: none"><li>• Preparing for NICE committee meeting for a challenging topic</li></ul>	<ul style="list-style-type: none"><li>• Confident advocates at NICE meetings who reported feeling well prepared for the meeting and for the following stages of NICE process</li><li>• Impact of patient voice highlighted in NICE guidance</li><li>• Positive NICE guidance</li></ul>




## Case Study 3: Presenting evidence for impact

<b>Who</b>	<b>Support</b>
<p>Small rare cancer patient organisation Run by the daughter of someone who died from the condition with 1-3 part-time staff/volunteers Treatment options available for early disease but not for metastatic disease HTA process underway – submission due in 3 weeks</p>	<p><b>Evidence review and generation</b></p> <ul style="list-style-type: none"><li>• Reviewed available evidence and identified key themes</li><li>• Identified topics and questions for PAG to use with small number of interviewees</li></ul> <p><b>Presenting evidence for impact</b></p> <ul style="list-style-type: none"><li>• Ensuring PAG understood purpose of the submission and what was in and out of scope for the committee</li><li>• Identifying key issues for inclusion</li><li>• Drafting and review of submission</li></ul>
<b>Need</b>	<b>Impact</b>
<ul style="list-style-type: none"><li>• Urgent support to maximise impact of limited evidence in a NICE submission due 3 weeks from first contact</li></ul>	<ul style="list-style-type: none"><li>• Submission well received by NICE committee</li><li>• PAG content that all aspects of disease burden captured in the draft guidance</li></ul>

## Case Study 4: Scoping to submission

Who	Support
<p>Small ultra rare disease patient organisation</p> <ul style="list-style-type: none"> <li>• 1 part time volunteer also runs own business. Parent of child with the ultra rare condition.</li> <li>• No licensed treatment</li> <li>• Scoping stage of NICE process with no prior involvement in clinical trial design</li> <li>• 3 companies with an interest in the condition</li> </ul>	<p><b>Structured module</b></p> <ul style="list-style-type: none"> <li>• Introduction to HTA</li> </ul> <p><b>1:1 coaching</b></p> <ul style="list-style-type: none"> <li>• Focus on action planning and funding</li> </ul> <p><b>Additional support</b></p> <ul style="list-style-type: none"> <li>• Guidance and support on responding to draft scope(written response and scoping meeting)</li> <li>• Community Survey design and delivery</li> <li>• Guidance and support to present evidence for maximum impact</li> </ul> <p><b>Tools</b></p> <ul style="list-style-type: none"> <li>• Preparedness checklist</li> <li>• Action planning template</li> <li>• Funding application template</li> </ul>
Need	Impact
<ul style="list-style-type: none"> <li>• Understanding the HTA process and how to effectively participate</li> <li>• Generating an presenting evidence to maximise impact</li> <li>• Generating funds and increasing resources to ensure charity is able to effectively engage in HTA</li> </ul>	<ul style="list-style-type: none"> <li>• Confident advocate at NICE meetings</li> <li>• Final scope reflected patient views</li> <li>• Funding application submitted</li> <li>• Survey underway to fill evidence gaps for submission</li> </ul>

# Case Study 5: Building collaborations

Who	Support
<p>5 UK PAGs for a rare disease with different views on how to engage with the HTA process</p> <p>Rare disease with one treatment available for some patients</p> <p>All PAGs had some experience of HTA but not the skills and understanding to maximise their impact</p>	<p><b>Agreeing an approach</b></p> <ul style="list-style-type: none"> <li>Facilitating discussion to identify lead PAG to coordinate evidence generation and submission</li> <li>Agreeing key topics to include in the submission</li> </ul> <p><b>Managing evidence generation and presentation</b></p> <ul style="list-style-type: none"> <li>Managing conflicting views about what to include and the approach needed.</li> <li>Working with a pre-selected vendor to ensure community survey design addressed priority topics for HTA</li> <li>Drafting and reviewing survey report – presented so that it could be used as the body of a NICE submission</li> </ul>
Need	Impact
<ul style="list-style-type: none"> <li>Aligning on approach to NICE submission</li> <li>Generating and presenting evidence for impact</li> </ul> 	<ul style="list-style-type: none"> <li>Described by one experienced pharma reviewer as best PAG submission they had seen</li> <li>All 5 PAGs content with submission as a reflection of their views</li> <li>Note: appraisal cancelled due to product not receiving Marketing Authorisation</li> </ul>

# Case Study 6: Managing community expectations



<b>Who</b>	<b>Support</b>
<p>Rare disease with two UK disease specific PAGs, one UK umbrella group and one US based PAG</p> <p>Limited treatment options and no experience of HTA</p> <p>Active wider patient community with high expectations for new treatment</p> <p>HTA process underway –scoping stage</p> <p>Additional treatments in development</p>	<ul style="list-style-type: none"><li>• Facilitating discussions between PAGs to agree approach to scoping consultation</li><li>• Advice and guidance in preparing for scoping workshop</li><li>• Advice on content for community events to explain process</li><li>• Advice on preparing for submission and committee meetings</li></ul>
<b>Need</b>	<b>Impact</b>
<ul style="list-style-type: none"><li>• Understanding the NICE process, how to work together and how to engage the wider patient community</li></ul>	<ul style="list-style-type: none"><li>• Agreement between the PAGs on how to work together</li><li>• Agreement on key messages to patient community</li></ul>

