## Worksheet for the development of patient-reported outcomes (PRO) and clinical outcome assessment (COA) strategy

Crafting a comprehensive Patient-Reported Outcomes (PRO) and Clinical Outcome Assessment (COA) strategy is a nuanced endeavor. It requires careful consideration of numerous critical factors, including regulatory requirements, and the needs of other stakeholders such as of Health Technology Assessment (HTA) bodies, clinicians, and patients. The complexity of the process necessitates meticulous planning and expert insight.

To assist you in this process, you can download a detailed worksheet, developed by Sarah Donelson, Kate Williams, Sarah Acaster, and Jenya Antonova. This tool is designed to guide you in formulating an effective and thorough PRO and COA strategy.

The tool consists of multiple questions, answers to which can help you shape your strategy. There is no right or wrong answer. Each product strategy is unique and requires unique approach to planning PRO and COA strategy.

The tool comprises several sections we recommend answering as many questions as possible in each section. This will help ensure robust and comprehensive (as opposed to one-sided) approach to the strategy.

We hope this tool can be of assistance to you.

*Key disease and product characteristics*

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| What symptoms does the disease cause?<br><br>Which of these are likely to be affected by the treatment?<br><br>In what direction and to what extent this effect is expected? | |
| What symptomatic adverse events can the treatment cause? How severe are they expected to be? | |
| What is the expected effect of the treatment on<br>- Health-related quality of life in general<br>- Physical Functioning<br>- Social Function<br>- Productivity<br>- Caregivers and other family members (if applicable)<br>- Other aspects of HRQoL? | |
| How quickly is treatment expected to start working? | |
| Considering the same questions as above, how is the product expected to be differentiated from the competition? | |

*Regulatory considerations*

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| Which agencies are primary stakeholders for your regulatory strategy? What are their requirements? | |
| Which product characteristics listed above are likely to be relevant within regulatory context? | |
| Is labeling language desired for COA endpoints? | |
| What are regulatory requirements and precedence in this disease area and therapeutic space. | |
| What data are need to be generated to support regulatory review? | |
| What steps do you propose to reduce uncertainty and de-risk regulatory strategy? | |
| Is there anything relevant for regulatory strategy? | |

*HTA and payer considerations*

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| Which agencies are primary stakeholders for your HTA and payer strategy? What are their requirements? | |
| Which product characteristics listed above are likely to be relevant within HTA and payer context? | |
| What should be your HTA and payer strategy? | |
| What steps do you propose to reduce uncertainty and de-risk the HTA and payer strategy? | |
| Is there anything relevant for the HTA and payer strategy? | |

*Policy considerations*

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| Which what other stakeholders can affect your access strategy? | |
| Which policy makers may be decision makers or stakeholders? | |
| Which patient advocacy organizations exist, and what are their priorities?<br><br>Can your product or a program help them in achieving their priorities? | |
| Which product characteristics listed above are likely to be relevant to these stakeholders? | |
| What should be your strategy for these stakeholders? | |
| What steps do you propose to reduce uncertainty for this strategy? | |
| Is there anything relevant for these stakeholders? | |

*Overall summary*

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|---|---|
| What should be your overall all-stakeholder strategy? | |
| What should be done and at what stages of the product lifecycle? | |
| What is your publication strategy? | |
| What are other important considerations you would like to highlight? | |