Effective Engagement Between Sponsors & Patient Groups: A Structured Process from the Clinical Trials Transformation Initiative (CTTI)

Background
CTTI is developing an open-access, web-based “Prioritization Tool” to assist sponsors and patient groups with identifying high-value opportunities to collaborate. The tool incorporates a simplified, 3-step decision-making process incorporating insights from semi-structured interviews, pilot-testing, and multi-stakeholder project team discussion.

Results
Updated Engagement Activities
Building on prior project work and incorporating feedback from semi-structured interviews, CTTI developed a refined list of patient group engagement opportunities. Figure 1. The Prioritization Tool will provide this list as a starting point for discussion between research sponsors and patient groups that are interested in working together.

Evaluating Potential Benefits and Investments
The interview findings identified factors and examples to consider when estimating the potential benefits and investments associated with engagement opportunities. Table 1.

Table 1: To identify high-value opportunities for research sponsors and patient groups to work together, consider:

<table>
<thead>
<tr>
<th>Potential Benefits</th>
<th>Potential Investments</th>
</tr>
</thead>
</table>
| Scope of impact on patient population or organization | Financial resources needed
For example, some patient group engagement activities might have potential to impact a large segment of the patient population or several future trials. |
| Necessity of patient group involvement | Staff time and expertise required
For example, some research activities might only be possible with the involvement of patient groups. |
| Necessity for advancing medical product development | Potential patient burden
For example, some patient group engagement activities might be critical to conduct in order to advance development of a medical product. |
| Reputational benefits | Additional organizational commitment needed given existing infrastructure
For example, some patient group engagement activities might demand a great deal of commitment from the organization to establish necessary infrastructure and processes. |
| Reputational risks | For example, some patient group engagement activities might pose a potentially serious risk to the reputation of the sponsor or patient group if they are carried out or not done well. |

Figure 1. Patient Group Engagement Across the Clinical Trial Continuum*
Patient groups have potential to enhance the quality and efficiency of clinical trials by providing:

- Financial support for research
- Natural history data
- Input on relevance of research to patients
- Access to translational tools
- Help defining eligibility criteria
- Input on meaningful endpoints & PROs
- Advocacy for policy & funding issues
- Education to patient community

- Support to sponsors around key regulatory meetings
- Support preparing submissions for newborn screening for rare diseases
- Informing regulators on benefit-risk
- Public testimony at regulatory meetings

Discovery & Preclinical†
Phase 1–3
Regulatory Review
Postapproval

- Benefit-risk & patient-preference studies
- Protocol design & study feasibility input
- Study recruitment & retention strategy input
- Increased awareness about trials
- Participant feedback on trial experience
- Input on informed consent content & processes
- Peer advocates for participants
- Clinical trial networks
- Data and Safety Monitoring Board members

Phase 1-3 activities and...
- Support interpreting & disseminating study results
- Collaboration on post-marketing studies & surveillance initiatives
- Support developing access strategy & preparing for value or health technology review

*Updated 2018; adapted from Parkinson’s Foundation materials | †Patient group activities typically undertaken independently or with partners other than sponsors | ‡Includes early planning for trials
To learn more about CTTI’s Patient Groups & Clinical Trials work, please visit [https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials](https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials).

**Prioritization Process**

**Identify RELEVANT engagement activities**
- Starting with their own list and/or the list of engagement activities above, patient groups and research sponsors—working either together or independently—will filter down to those that are relevant to the situation of interest.
- Users will be provided examples of each activity and encouraged to identify their own fit-for-purpose implementations.

**Evaluate BENEFITS and INVESTMENTS associated with each activity**
- For each relevant engagement activity, users will rate likely investments and benefits as High, Moderate, or Low.
- Evidence-based factors identified by CTTI (see Table 1) will be provided as a starting point for consideration.

**Identify MUTUALLY BENEFICIAL activities**
- This process generates a Priorities Matrix (Figure 2) identifying the relevant activities that have the highest “value” (benefit vs. investment).
- Research sponsors and patient groups interested in working together can compare and discuss priorities to arrive at opportunities that are of high value for each (Table 2).

**Priorities Matrix:** The Prioritization Tool assists research sponsors and patient groups in quickly prioritizing relevant activities based on benefits vs. investments. Figure 2.

**Example Joint Prioritization Outcome:**
Table 2 shows an example of how a patient group and research sponsor might assess three activities identified as relevant to their potential collaboration.

**Table 2. Example Joint Prioritization Outcome**

<table>
<thead>
<tr>
<th>Engagement Method</th>
<th>Impact on Value Drivers</th>
<th>Patient Group</th>
<th>Sponsor</th>
</tr>
</thead>
<tbody>
<tr>
<td>Input on relevance of research to patients</td>
<td>Facilitating benefit-risk assessment, focus groups, and survey studies requires small investments for patient groups and industry sponsors while the potential benefits of this information are great. The resulting information may improve study designs, leading to shorter study durations, lower risk, and lower costs.</td>
<td>High benefit / Low investment</td>
<td>High benefit / Low investment</td>
</tr>
<tr>
<td>Help defining eligibility criteria</td>
<td>Assisting in the definition of study eligibility criteria requires little investment for patient groups and sponsors but may have great benefit by more precisely defining the study population, providing a potential to improve the study design and reduce study duration, costs, and risks.</td>
<td>High benefit / Low investment</td>
<td>High benefit / Low investment</td>
</tr>
<tr>
<td>Financial support for research to identify target molecules</td>
<td>Funding target molecule identification requires a significant investment for patient groups with low probability of success. However, the benefit-to-investment ratio is larger for sponsors due to the minimal investment required on their part even if the likelihood of success is low.</td>
<td>Moderate benefit / High investment</td>
<td>High benefit / Low investment</td>
</tr>
</tbody>
</table>

**Contact details**

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