THE CENTER FOR RARE DISEASES

Guiding Principles for Interactions with Rare Disease Patient Advocacy Organizations

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The Center for Rare Diseases believes that when patients are truly partners in the clinical development process, clinical trial design and execution are enhanced through inclusion of specific and meaningful quality of life outcomes, and successful strategies to minimize participation burden and maximize study awareness and education.

Center for Rare Disease Mission Statement: We are guided by patients who understand better than anyone else that rare disease alters entire lives. We are transforming clinical research into meaningful healthcare options through patient partnerships, data analytics, and technology solutions.

The CRD believes in holding our interactions with patient advocacy organization to the highest possible standards. This document is meant to serve as a basis for understanding the way the Center for Rare Disease at PRA will conduct interactions with patient advocacy organizations.

*For the purposes of this document, the term Patient Advocacy Organizations (PAO) will encompass rare disease specific organizations, umbrella rare disease patient organizations, research foundation, nonprofit organizations supporting rare disease and support groups or networks supporting rare disease.

The guidelines in this document have been informed by:

- Stein et al. Orphanet Journal of Rare Diseases (2018) 13:18 DOI 10.1186/s13023-018-0761-2
- The International Fibrodysplasia Ossificans Progressiva Association (IFOPA) Guidelines for Engagement with Pharmaceutical Companies. V1.0; Casselberry, FL: IFOPA. 2016.
- Clinical Trials Transformation Initiative (CTTI). Effective Engagement with Patient Groups Around Clinical Trials. 2015.
- European Federation of Pharmaceutical Industries and Associations. EFPIA Code of Practice on Relationships Between Pharmaceutical Companies and Patient Organisations. Initially approved 2007; amended and adopted June 14, 2011.

The Center for Rare Diseases has identified three main areas of focus under which our guiding principles live. These areas are:

1. **Trust-** holding the patient/PAO's trust and privacy to the highest standard
2. **Communication**- prioritizing effective communication and engagement
3. **Partnerships** prioritizing *meaningful* partnerships.

Trust

The trust of patients and patient advocacy organizations is the foundation in which meaningful partnerships can be built. Trust develops when the privacy and confidentiality of patient advocacy organizations is held in the highest regard. The following guiding principles illustrate our perspective on how we regard the privacy and trust of patients.

1. **Hold Patient Privacy to the Highest Standard**
   1.1. PRA will hold the privacy and trust of the PAOs above all else. PRA will never record, release, or distribute any personal health data without proper informed consent
2. **Follow Through on all Commitments**
   2.1. PRA will honor all commitments made to PAOs to the best of our ability
3. **Honor Confidentiality**
   3.1. Clearly explain and demonstrate that PRA does not share, sell or distribute any information about patients/patient advocacy groups without their explicit consent

Communication

Communication should be transparent, thorough and take place often. Communicating and engaging with patient advocacy organizations is an essential component of ensuring successful drug development program. Communication and engagement should begin at the earliest possible point and continue long after the completion of a clinical trial. The following guidelines reflect the standards of PRA's Rare Disease Team.

1. **Engage with the Patient Organizations at the Earliest Possible Junction**

   1.1. Encourage sponsors to engage (or allow PRA to engage on their behalf) at the earliest point possible in the drug development program

   1.2. PRA will engage with PAOs at the soonest possible junction to increase the value that patient organizations can add to a clinical development program

   1.3. How/when PRA will engage with PAOs will be based on where the program is currently in its development

   1.4. PRA will engage with PAOs in the manner established as most appropriate by the PAO as well as the sponsor

   1.5. PRA will assist the sponsor in understanding how patient engagement can take place and how it can be most effective

2. Communicate transparently and Effectively

2.1. Clearly define the role of the CRO & sponsor, responsibilities and expectations that both the PAO and PRA have for the partnership

2.2. Clearly define and understand the expectation the PAO has for the Sponsor Company and how the PAO is willing to work, directly or indirectly with the sponsor company as well as the expectations the sponsor company has for the PAO.

2.3. PRA will regularly communicate with PAOs even when there is no "new" study news to ensure the partnership grows and deepens

2.4. PRA will communicate information such as (but not limited to) study events, modifications, redirections, postponements and cancelations and study outcomes

2.5. PRA recommends clear, open, and transparent feedback between the PAO, the sponsor and the CRO during any and all collaboration

3. Engage on behalf of the best interest of the patient/patient advocacy organization

3.1. PRA will always prioritize the best interests of the patient(s) and PAO(s) when engaging

3.2. Each PAO (in a disease space) will have different capabilities and areas of expertise. PRA will work with individual groups to determine the most effective way of utilizing their unique capabilities

4. Communicate with all patient advocacy organizations within the community

4.1. PRA will actively work to engage each PAO within the disease community and not exclude any group

4.2. PRA understands that not all organizations will wish to engage, but that all should be offered involvement

4.3. PRA will not engage in any politics between PAOs

5. Define communication plans and preferences

5.1. PRA will establish communication plans based off the preferences of the PAO, the sponsor and the project timeline

6. Manage expectations and conflicts of interest

6.1. PRA will manage expectations through:

6.1.1. Transparent and frequent communication

6.1.2. The outlining of expectation of both PRA, the sponsor and the PAO regarding the study

6.2. PRA will work to identify, disclose and discuss any potential conflicts of interest

7. Provide patient advocacy organizations with a "service agreement" if appropriate or requested

7.1. PRA will provide PAOs with a Service Agreement or mutual letter of understanding (MLU) to identify the roles and responsibilities of each party, when appropriate

8. Provide updated engagement plans/strategies

8.1. PRA understands that plans & strategies can change and may require evolution

8.2. PRA will provide PAOs and sponsors with updates to the engagement strategy and plan as they arise

8.3. PRA will work with the PAOs and sponsors to ensure that the engagement strategy remains current, applicable and effective

9. Provide education and support to patient organizations whenever possible/appropriate

9.1. PRA will provide education and resources to PAOs regarding the clinical trial process, expectations, accessing trials etc. when appropriate

9.2. PRA will provide educational or support resources to the sponsor when appropriate in effort to increase the efficacy of the program

Partnerships

The PRA Center for Rare Diseases believes that a meaningful partnership is a key factor in the success of any drug development program in rare disease. Meaningful partnerships are built and grow over time. Meaningful partnerships are built on trust, transparency, mutual benefit and continued and deliberate engagement.

1. **PRA encourages the establishment of a relationship (leading to partnership) between patient advocacy organizations and sponsor and help facilitate engagement when appropriate**
2. **PRA will always act on behalf of the patient advocacy organization's best interest**
3. **PRA will act as a conduit between patient advocacy organizations and sponsor when appropriate**
4. **PRA encourages and will help establish/maintain a mutually beneficial partnership where all parties have a stake in the game**
5. **Provide patient advocacy organizations with resources/assistance (where/when appropriate) to aid in the growth and development of the patient advocacy organization**

5.1. Provide documented accounts, to both the PAO and sponsor, on the *Return on Engagement* with patient advocacy organizations