

Effective Patient-Focused Drug Development: An Example Based On People With Multiple Sclerosis Collaborating With A Pharmaceutical Company

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BACKGROUND

- The U.S. FDA is undertaking an initiative, *Patient Focused Drug Development (PFDD)*, to facilitate a systematic integration of patient perspectives in drug development and evaluation processes, under the 21st Century Cures Act and PDUFA VI [1,2]. Similar initiatives are underway globally [3].
- In 2018, EMD Serono entered into a PFDD collaboration with Accelerated Cure Project/iConquerMSTM People-Powered Research Network to capture and integrate perspectives of people with relapsing forms of multiple sclerosis (RMS) into RMS drug development. A PFDD Council including people with RMS has been created towards this end.

OBJECTIVES

- To describe the practical steps in the establishment and implementation of EMD Serono and ACP/iConquerMSTM PFDD collaboration
- To describe the initial experience of people with MS participating in the PFDD Council and to identify factors that are key to a successful PFDD collaboration

METHODS

- The main considerations in the set-up and implementation of the PFDD collaboration were based on current best practice and frameworks on patient-engagement [3, 4]. The steps followed in implementing the collaboration were agreed between EMD Serono and ACP/iConquerMSTM (Figure 1).

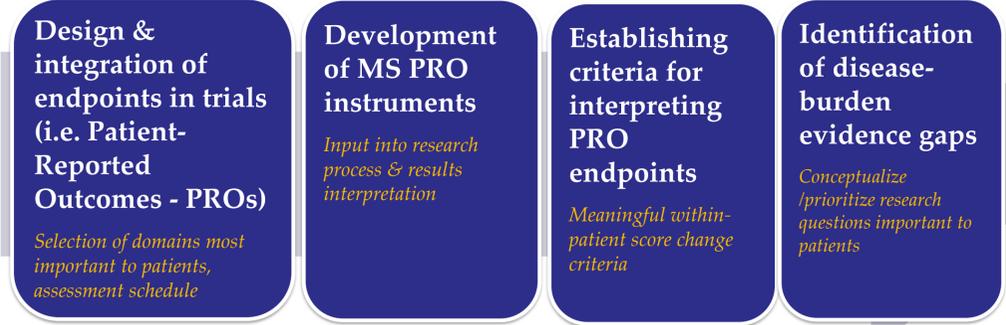
Figure 1: Implementation steps for PFDD collaboration

- Step 1 Definition of objectives and priorities
- Step 2 Joint scoping exercise
- Step 3 Development of program of work
- Step 4 Perform internal governance and ethics reviews
- Step 5 Mapping and engagement with internal stakeholders
- Step 6 Incorporate insights into development program

RESULTS

- At the outset, four priority areas were jointly identified by EMD Serono and ACP/iConquerMSTM for the collaboration (Figure 2).
- A council of people with MS (n= 8) was established to serve as a standing panel, providing patient perspectives and driving the priority activities.
- Various channels were used to obtain feedback from the PFDD Council including *face-to-face workshops, regular teleconference, structured surveys, review of study plans and results reports.*

Figure 2: Priority areas for PFDD collaboration



Key success factors of the PFDD initiative

- Modus Outcomes conducted semi-structured interviews of the PFDD Council members to obtain their first-hand experience of the EMD Serono & ACP/iConquerMSTM collaboration.
- Thematic analysis of interview transcripts identified four core aspects that contributed positively to the success of the council (Figure 3). Example quotations are presented in Figure 4.

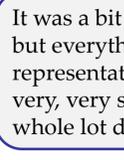
Figure 3: Success Factors for PFDD collaboration

1. Clear expectations and understanding of roles
2. Appropriate skills and training to support role
3. Positive and collaborative mindset, values, and behaviors of all persons involved (i.e. EMD Serono, ACP/iConquerMS, and the PFDD council members)
4. Open and quality communication

Figure 4: PFDD Council member quotations



I think and feel that my role is to provide feedback and information and insights to what is to live with MS and what you go through on daily basis... What the symptoms and the relapses and the impact of our symptoms over time, how they change. So just give that personal insight to gather that information, so that they can make an informed decision about the drug. It's just a great opportunity to be able to be a part of something so great... (#1)



It was a bit daunting not really knowing what I was jumping into, but everything became so well defined, whether it's the representatives from EMD or Accelerated Cure, there's been a very, very strong focus on hearing what we had to say, which is a whole lot different, then giving us a chance to talk (#3)



I'm of the mindset that even if there's no news to tell, we need to say there's no news. That we don't leave people just wondering what's next. Where are we at with this. I think that's really, really important and I would love to see more frequent contact via email (#4)

CONCLUSIONS

- Successful PFDD collaborations require beginning with the end in mind; by defining clear objectives and priorities as well as planning how insights will be integrated within development programs.
- This PFDD collaboration reflects a paradigm shift in patient engagement during drug development from a transactional engagement model towards a more transformative patient partnership approach to drug development.
- Collecting meaningful patient experience data is a necessary beginning step to ensure a patient-focused approach, yet, organizational change management is essential to fully realize the full potential of patients focused drug development.

REFERENCES

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