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/iConquerMS™ People-Powered Research Network to capture and integrate perspectives of patients 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/iConquerMS™ 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/iConquerMS™ (Figure 1).

RESULTS

- At the outset, four priority areas were jointly identified by EMD Serono and ACP/iConquerMS™ 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 1: Implementation steps for PFDD collaboration

Figure 2: Priority areas for PFDD collaboration

Design & integration of endpoints in trials (i.e. Patient-Reported Outcomes - PROs)

- Identification of outcomes most important to patients, treatment schedules

Development of MS PRO instruments

- Input into research process & results interpretation

Establishing criteria for interpreting PRO endpoints

- Meaningful evaluation, patient score change

Identification of disease-burden evidence gaps

- Enriched patient experience research priorities

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/iConquerMS™ 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: Key success factors of the PFDD initiative

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