Platelet Disorder Support Association

20 Years

Empowering ITP Patients

Education. Advocacy. Research. Support.

ENGAGING WITH THE FDA

1998

PDSA Founded

2008

ITP Patients, PDSA Medical Advisors testify to FDA (ODAC) for approval of two new ITP therapies

2016

PDSA receives grant from NORD via the FDA for ITP Natural History Study Patient Registry

Attendance at 1st FDA Public Workshop

2017

ITP Registry Launches

FDA Workshop #2

FDA Workshop #3

Meeting with OHOP/PASE

2018+

FDA Workshop #4

FDA Workshop #…

- *Externally-led PFDD?*
- *Submission of Registry data?*
- *Further testimonies for new ITP therapies?*
- *Comments on Draft Guidances?*

PLANNING YOUR MEETING

1. INVOLVE KEY OPINION LEADERS: patients and caregivers, patient advocacy group, physicians, researchers
2. CLEARLY ESTABLISH GOALS:
    1. Educate the FDA on the most significant symptoms, current treatment side effects, burden of disease, and impact of condition on daily life.
    2. Ensure that the patient voice is included in providing guidance and advancing science.
    3. Serve as a comprehensive resource on the patient experience to provide input and guidance in new drug development research moving forward.
3. DEVELOP YOUR ASK: PRIORITIZE THE UNMET NEEDS OF PATIENT POPULATION
4. PROVIDE PATIENT EXPERIENCE DATA

TAKE-AWAYS: BENEFITS OF COLLABORATING WITH THE FDA

> *"Meetings are greatly enriched by the inclusion of patients with the condition… they provide the most valuable insights"- Theresa Mullin, Associate Director for Strategic Initiatives, CDER (3/19/18)*

- **Involvement of all stakeholders**
- The FDA wants to include the patient perspective: help them to help you
- Have the right people in the room and ask the right questions: identify issues up-front that FDA should be addressing to maximize impact of meeting
- Encouraging to patient population that advocacy groups are collaborating with the agency
- Patients are able to express what matters most to them and take charge of their health
- Advocacy work is never done, follow up!

BENEFITS OF ENGAGING EARLY AND OFTEN: ACCESS!

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Future opportunities to express to FDA what matters most to patients