Mapping outcomes to coreVWD: moving toward a fully reportable core outcome set to improve access to treatment for people with von Willebrand Disease (VWD)

E Clearfield,[1] E Ayoub,[2] B Ziemele,[3] M Delaney,[4] T Youttananukorn,[2] D Coffin,[2] F Brennan,[1] A Kucher,[1] MW Skinner,[1] P James[5]

1. Patient Outcomes Research Group, Washington, DC; 2. World Federation of Hemophilia, Montreal, Quebec, Canada; 3. Latvia Hemophilia Society, Riga, Latvia; 4. National Bleeding Disorders Foundation, New York, NY; 5. Queen's University, Kingston, Ontario, Canada

BACKGROUND

The VWD treatment landscape is expanding; to assess upcoming products for efficacy and cost-effectiveness, outcomes must be harmonized across clinical trials, registries, and post-market studies. The coreVWD Initiative (Clearfield, *Haemophilia*, 2024) convened a multi-stakeholder group to align on a recommended core outcome set (COS) important to decision-makers across the product lifecycle. Outcomes were prioritized for prophylaxis, perioperative treatment, and for women, girls, and people with the potential to menstruate (WGPPM); adverse events were also considered (Figure 1). To fully utilize these recommendations, we must now identify how to most efficiently measure, collect and report them.

AIMS

Using coreVWD as a benchmark, to map the core outcomes identified to those currently collected in prominent ongoing studies and identify points of overlap and gaps to be covered by updated or new measurement tools.

METHODS

We compared the coreVWD COS to the World Federation of Hemophilia's World Bleeding Disorders Registry (WBDR) and myWBDR app datasets and to variables collected in the Patient Reported Outcomes Burdens and Experiences (PROBE) Study, a quality-of-life study for people with hemophilia.

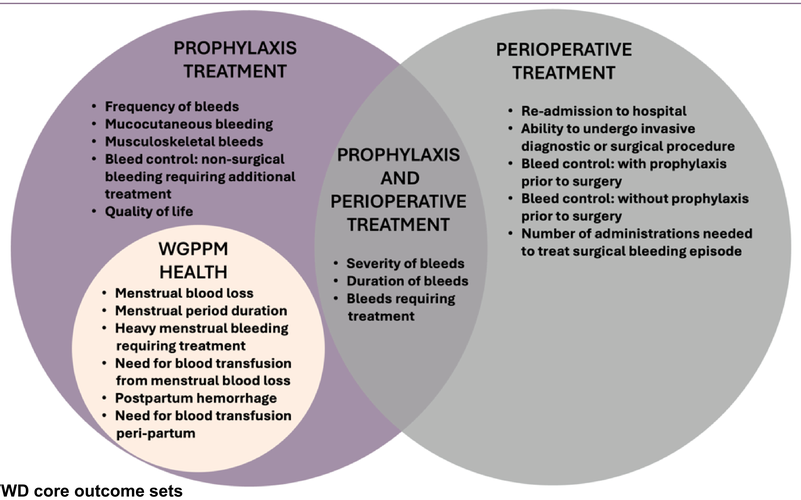

Figure. The coreVWD core outcome sets

Table. Mapping of coreVWD Outcomes to WBDR, myWBDR, and the PROBE Study Data Elements

Empty cells represent data gaps. Grayed cells indicate partial coverage or that outcome can be derived from data dependent on type of assessment used and/or detail provided in open text boxes. [1]PROBE is collected as part of the myWBDR PRO module or can be administered independently; [2]BAT Scores assessed by Self-BAT; [3]BAT Scores assessed by ISTH BAT; [4]BAT Scores assessed by MCMDM-1; [5]PROBE is a quality-of-life study, further description of what should be included as QOL for those with VWD is needed; [6]Additional WGPPM PROBE Study elements are currently undergoing field testing.

RESULTS

Frequency of bleeds and bleeds requiring treatment are well covered by WBDR, myWBDR and PROBE, but other descriptions of bleeding are only captured dependent on what Bleeding Assessment Tool (BAT) is used (Table). Information about perioperative bleed control is only collected in WBDR. All three tools could better cover the core outcomes if they enhanced or added a section for WGPPM on menstrual bleeding and pregnancy experiences.

CONCLUSIONS

coreVWD prioritized outcomes important to patients; the coreVWD outcomes will require both patient and clinically reported data. Presently, they are not fully captured using currently in-use tools. Updates that aim to include as many core outcomes as possible will allow for WBDR, myWBDR, and PROBE to serve as important data collection tools to bring the appropriate treatments to people with VWD.

 For more information contact: eclearfield@ipaltd.com