

COSEB Workshop 2024 - Towards Harmonization of Outcomes in EB

The COSEB (Core Outcome Sets for Epidermolysis Bullosa) workshop was held as a hybrid meeting in Amsterdam, The Netherlands on December 15th 2023 and organised by the COSEB Steering committee and DEBRAs of Austria, France, Ireland, Spain, and the United Kingdom. The goal of the COSEB initiative is to establish consensus-based core outcome sets for the major EB types (EB simplex, junctional EB, and dystrophic EB) by identifying the most critical outcome domains (the “what” to measure) and corresponding outcome measurement instruments (the “how” to measure). Such core sets should be used and reported consistently across clinical trials, thereby facilitating accurate comparison and pooling of data to expedite therapy development.

Funded by the European Joint Programme for Rare Diseases (EJP-RD), the workshop aimed to foster collaboration among key stakeholders in EB research to address challenges concerning outcome measurement in EB. Specifically, its objectives included: 1) to inform all stakeholders about the methodological steps of the COSEB process, 2) to connect working group members for planning activities for the upcoming year, and 3) to assess the feasibility of the COSEB protocol and appropriateness in light of available resources to meet the needs of the working groups. A total of fifty attendants from Europe, North and South America, Australia and Asia participated in the meeting, consisting of patient advocates (n=7) and EB professionals including clinical experts (n=33), industry partners (n=3), regulatory representatives (n=3) and methodologists (n=4), that signed up for a working group or advisory panel of COSEB.

The workshop commenced with a presentation of the COSEB protocol by Eva Korte and Martin Laimer, both members of the COSEB founding and steering committee. They outlined the first methodological steps of getting to a short-list of candidate outcome domains, as well as the organizational structure of the research teams, including the responsibilities of the steering committee, advisory panels and working groups. The workshop continued with explanations regarding the Delphi consensus procedure and granularity of outcomes by COS methodologists Sanna Prinsen and Jan Kottner, respectively. They emphasized the value of a consensus-based standardization of trial outcomes and underscored the importance of balancing the level of granularity and specificity when defining core outcome domains. Subsequently, Gaston Sendin from DEBRA Austria addressed the priority of getting functional and clinically meaningful outcomes and trial endpoints. He pointed the need for collaboration among research, industry, and organizations like DEBRA to secure funding for initiatives like this. The session continued with an introduction on the consensus method of COSEB and a section on the modality to engage patients in the initiative. The moderators of the session, Peter van den Akker and Sagair Hussain, summarized the first plenary session by highlighting the importance of valid and feasible core outcomes, accurate granularity, and broad stakeholder engagement with emphasis that active involvement from the entire EB community is critical to the success of COSEB.

Subsequently, in two separate break outs, the members of the dystrophic EB and EB simplex working groups were introduced and connected. Chairs and deputy chairs were elected and a first inventory of questions and requirements for the COSEB protocol was discussed. The final plenary Q&A session together with COS methodologists, Jan Kottner and Phyllis Spuls was intended to clarify any open COS related questions. In their closing remarks, the members of the steering committee summarized once more the upcoming tasks and responsibilities of the working groups and advisory panels. Particularly, the timely submission of the Conflict of Interest forms by all participants in addition to the provision of a structured feedback on the COSEB protocol per working group was requested (before February 1st 2024 to coseb@umcg.nl). Other milestones shared during the meeting included: February 6th for a follow-up meeting on protocol and methodological approach with the working group chairs, February

22nd for a Q&A session on any open questions/issues for all participants, and March 1st to prepare a working plan and budget request for each working group.

In conclusion, the COSEB workshop focused on the collaborative efforts to establish core outcome domain sets for EB trials, aiming to enhance patient care and accelerate clinical translation of therapeutic innovation for EB patients worldwide.