



17th Annual Scientific Meeting

Orphan Diseases
Working Group

Chairs:

Joan Busner, PhD

Gahan Pandina, PhD

Orphan Diseases Working Group Session at the 27th Annual ISCTM Scientific Meeting, April 6-9, 2021

Joan Busner, Ph.D. and Gahan Pandina, Ph.D., Co-Chairs

- Excellent attendance at this virtual meeting!
- Reviewed progress on our endpoints best practices manuscript; thanks to hard work of the manuscript subgroup, the manuscript is almost ready to be submitted for publication
- Discussed second planned manuscript on stakeholder input/improving patient access. Manuscript is in early phases and this is a good time for members to let the group if they would like to contribute to the effort
- Discussed plan to generate 2020 Autumn pediatric meeting report for publication (brief abstracts from each of our pediatric/orphan disease/patient centricity live sessions)
- Discussed future directions –
 - Another ½ day ISCTM live session – content ideas?
 - Clinical / regulatory approaches to de-risk clinical development
 - Examples:
 - Development program from start to finish (could use DMD as example)
 - How do we improve upon the status quo?
 - How little is too little? Minimum data requirements for endpoint ‘validation’ in rare diseases (again, with a detailed examples)
 - Other ideas welcomed!!