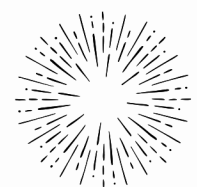
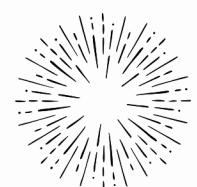


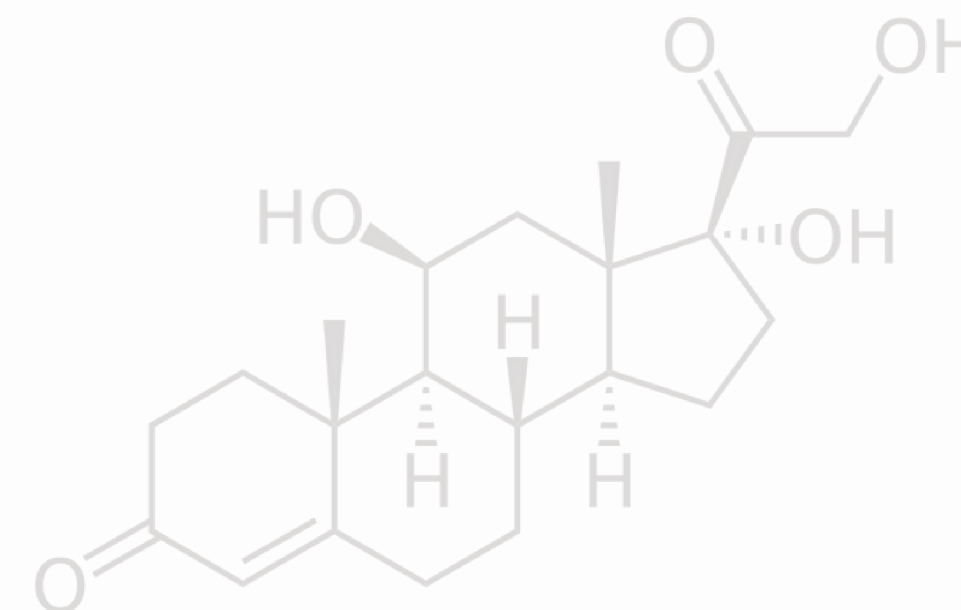
THE VALUE OF PEER COLLABORATIVE DATA COLLECTION in a rare disease with no specific symptoms



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Who Benefits, and Why Does It Matter?

PATIENTS

**DOCTORS,
SCIENTISTS**

**PHARMA,
BIOTECH,
INVESTORS**

**GOVERNMENT,
REGULATORS,
PAYERS**



Considering quality of care, we each have “pieces of the puzzle”. Only when all parts are there and fit, is it **complete**.

Patients must be at the table during therapy development, regulatory decisions, and trial design.

PATIENTS

- Patients who work together to build custom surveys for their peer groups have full control of the questions being asked.
- We are not limited or restricted by institutional requirements that often stifle exploration of novel data collection.
- We are highly motivated to create change for ourselves and future patients.
- Trial and error is ok!!!! Learning from mistakes and applying new knowledge is excellent.
- It is validating and powerful to be part of a team that is finding answers and identifying solutions to needs that went unmet during our own journeys with the disease. Plus, you get the answers first!
- Dedication attracts collaboration by people with similar missions and varied skills.

**DOCTORS,
SCIENTISTS**

- Patients are not limited or restricted by institutional requirements that often stifle exploration of novel data collection.
- Advocacy groups need advisory boards, which is a way to have a hand in survey/trial design via review and advice without having to take on the logistics.
- Validated data generated by highly motivated patient investigators can fill in huge understanding gaps re: natural history of a disease, long-term effects, and other aspects that are difficult to capture in clinic.
- A more deeply educated provider with empathy is one of our community's biggest **MUST HAVES**. It's hard to look away from the brutal truth of what it's like to live with a rare disease.
- Adding "humanity" to data points improves quality of work.

**PHARMA,
BIOTECH,
INVESTORS**

- **You tell us;** what is the value of a well-organized and motivated community of rare disease patients who WANT to work with you, who WANT to give you the data that can take so long and cost so much to originate without help???
- Data from institutional registries does not explain how disease affects patients' lives. Your products will be better if they are attractive tools designed for real-world use rather than hyperfocused on FDA endpoints or other non-patient-created measures.
- Patient organizations who prioritize in-house research likely include their international peers in their work which can make starting up in other countries a lot easier if you can already identify a patient population to kick start a trial or study.

**GOVERNMENT,
REGULATORS,
PAYERS**

- Patient advocacy organizations can provide unique perspectives on proposed guidelines and trial design.
- Patient groups involved in research are usually operating at an elevated level of understanding of their condition and more likely to contribute to listening sessions, get involved in legislative advocacy, and participate in clinical trials.
- We postulate that educated patients get diagnosed and treated more efficiently, lowering costs for everyone involved.

DIDN'T GET ENOUGH? VISIT OUR BOOTH!

- MORE CONVERSATION
- NEWSLETTERS
- COPIES OF 2020, 2022, AND 2023 QUALITY OF LIFE STUDIES
- JOIN OUR MAILING LIST
- HOW CAN WE COLLABORATE?



ALSO JOIN US TOMORROW AT 2:15PM FOR **ROUNDTABLE 10: GRASSROOTS TO SCIENCE-READY: PATIENT CREATED RESEARCH**
TO CHAT ABOUT HOW WE BECAME READY FOR A PATIENT REGISTRY.