

The Paths to Trials Access for Rare Cancers - Patient Perspectives

Max Williamson

One Perspective



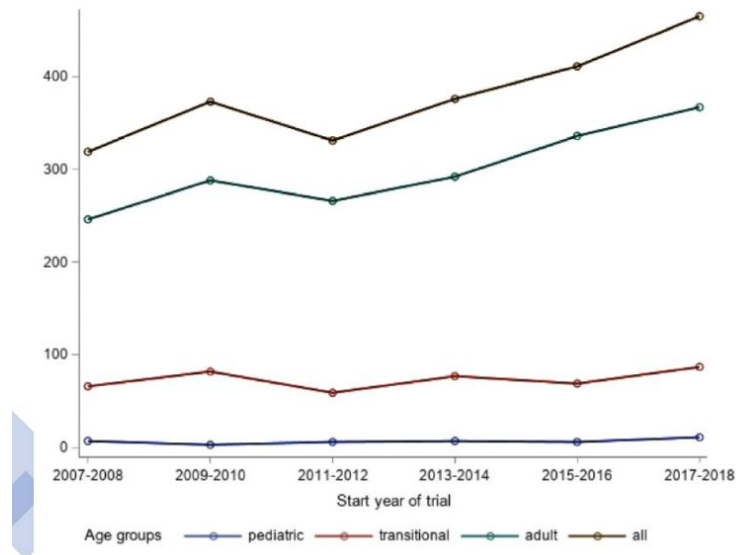
“Am I eligible?”

Age limits on clinical trials prevent access

T. De Rojas *et al.* (2019): TYA patients have limited access to clinical trials.

FAIR trials survey 2023: Only 35% of respondents aware of FDA guidance for 12-17 year old inclusion for trials.

FAIR trials group is working to analyse NA/ European pharmaceutical data



“How can I get involved?”

How can patient’s (and parents) support their own access to clinical trials?

Darmawan *et al.* (2020, JMIR): Across 33 reported studies (from >2700 titles), 7/20 studies had higher recruitment as a result of social media advertising

- For rare cancers, is it possible to use focussed patient targeting to inform patient’s of trial availability.

Actionable Challenge 3: Real World Evidence

“There aren’t any trials for me - can I still help others?”

In rare cancers, how can we extract as much evidence as possible from the patient populations available?

Rare cancer databases: can we begin to capture RWE and PROMs?

Tumour agnosticism: can pooled single arm data also capture PROMs?

Conclusions

- 1) Eligibility: Regulatory flexibility at the national, pharmaceutical and clinical level is the key to inclusion
- 2) Awareness: Patients and families want to be involved - awareness programs will be essential
- 3) Patient data: How can we be as resourceful as possible for rare cancer outcomes?