The Rare Disease Collective

What makes good clinical trials even better? The case for inclusivity.

Last week saw the 64th American Society of Hematology's annual conference (#ASH22) - the world's largest, championing the causes and treatments of blood disorders, including many rare diseases.

#ASH22 must surely have been the most diverse and inclusive ever, with a dedicated 'ASH Health Equity Studio' focused on issues such as *Overcoming Disparities in Patient Care, Addressing Implicit Bias and Overcoming Lack of Diversity in Clinical Trials*.

Yet, the hard realities of the main stages told another story. Presentations referenced papers showing clinical trials were hampered by inequality of participants - for example, African Americans make up 20% of multiple myeloma sufferers in the US, but account for less than 9% of individuals included in trials[1] and minorities, in particular black patients, being at a greater risk of being left behind on clinical trials of Diffuse large B cell lymphoma (DLBCL)[2].

Rigid clinical trial inclusion and exclusion criteria can rule out those with the highest mortality rates, enshrining unmet clinical need in research. In rare disease, clinical trial design is further complicated by small patient populations and patient types (e.g., ~50% of patients living with life-threatening rare conditions are children), a lack of sensitive (non-invasive) biomarkers and clinical outcome measures, and often poor understanding of the genotype–phenotype relationships and natural history, making clinical trial endpoints difficult to define.

Improving the inclusivity of clinical trials and other research does not just benefit under-served groups; it makes for better science. It supports the vital role of diversity in creating patient-defined outcomes, unmet need and patient inclusion in co-designing treatments, patient journeys and care pathways.

And a wider range of participants aids generalisation of results to a broader population. In doing so, we have a chance to make research more translatable to the clinic, to help overcome the bias and stigmatization that can exist with certain rare conditions, particularly those predominantly affecting African-American populations, such as sickle cell disease.

The existing development of treatments for rare diseases is a fragmented one - at the current rate, it would take more than 100 years to develop a single treatment for every rare disease estimated to exist worldwide.[3]

The Rare Disease Collective

We believe that we owe it to patients to think differently about clinical trial design. To adopt a more creative approach to designing research that is inclusive to those of all ages, ethnicities, physical and mental capacities – ultimately improving representation of the reality of the patient narrative and experience.

In doing so, we have an opportunity to embrace **who** patients are, not just what they have.

About the Authors:

n=1 is a unique agency collective, spearheaded by senior leads at agencies combining decades of expertise in rare disease from across advocacy and scientific communications, patient experience research and brand strategy.

Identifying unique needs across the lifecycle of clinical development and engagement in rare disease, **n=1** offers a tailored, targeted approach to tackling the challenges faced by those living with and treating rare diseases.

Enquiries:

Jon Linscott
jon.linscott@n-equals-1.com
m: +44 07736 125 028

To find out more, visit
n-equals-1.com

[1] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6192659/
[2] https://ascopubs.org/doi/full/10.1200/JCO.20.01935
[3] https://ec.europa.eu/research-and-innovation/en/horizon-magazine/building-blocks-make-rare-disease-treatments-more-common