

# Could COVID-19 be the Catalyst for Change in Future Rare Disease Research?

As we have just passed another Rare Disease Day, I wanted to concentrate on how clinical research can better support individuals and their families in the future. 300 million people suffer from a rare disease<sup>1</sup>. A tiny percentage of the whole population, but a significant figure, nonetheless.

Figures published in Tufts CSDD Impact Reports (2019)<sup>2</sup>, highlight the passion within the rare disease population to be involved in research, with dropout rates lower than in any other indication. Recruitment rates were higher across all indications than in previous years but with an average dropout rate of 19.1% across all indications it demonstrates how incredibly low rare disease dropout is, at just 6.5%.

You would expect these dropout rates would mean clinical trial design was perfect for these populations, but I worry this is not the case. Patients and families are so committed to trying to find treatments or support with their condition, it seems no length is too great. The most shocking example of this was an Icelandic family who were travelling to Canada for their trial visits. This usually involved at least a three-day round trip and all the family attending to manage the journey. In this example a specialist mobile research nurse was placed with the family who were considering dropping out as they simply could not cope with the travel any longer.

Fast forward a year and COVID-19 starts to take hold. Site visits halted, families left in limbo, managing rare diseases with little to no support and frightened of how COVID-19 might affect them. Suddenly, the clinical research landscape was forced into reform. If studies were to continue, alternative solutions would be required. That was where mobile research nursing came in, a service already growing in popularity within rare disease studies, but now brought to the front of sponsors' wish lists.

Mobile research nursing enables patients to complete some of their clinical trial visits in a location to suit them, generally in the home. Procedures such as bloods, vital signs, ECGs and abbreviated physical exams can be completed by a specialist skilled research nurse – a solution which offered a lifeline in keeping research going throughout the pandemic. Initially in some cases considered a short-term solution, but COVID-19 thought otherwise.

This service has been particularly successful within rare disease populations, as the distance to site becomes less of a factor when deciding to enrol. In one case study in Duchenne Muscular Dystrophy (DMD) all UK patients had to visit Great Ormond Street Hospital every week; challenging for a DMD patient, even if they live locally. An example of this was a family who before mobile research nursing support had a journey which involved over 12 hours of travel, including a ferry trip, none of which were reimbursed. The introduction of the service meant this challenging and tiring journey was reduced from weekly to monthly visits for the family, enabling them to continue in the study.

We still want to develop the service further to truly understand how patients with certain needs due to their conditions can best be supported. For example, in an indication like Epidermolysis Bullosa, how can we best minimise pain for these patients when they need to travel? When COVID-19 restrictions allow, we hope to be able to discuss solutions with advocacy groups and families to develop a truly patient-focused solution for their issues.

In the meantime, patient travel within COVID-19 studies is an area we have been supporting by offering vehicles with a dual ventilation system, keeping both the patient and driver safe when site visits are required. This is offered as a part of our patient concierge service, PatientGO®, which delivers patient travel, hotels and reimbursements. Again, an option which, alongside research nursing, sponsors will view as an option which improves the patient experience. Within the rare disease space, we see this as very beneficial in reducing the burden on families where travel and finances can become incredibly difficult, when a patient may have a debilitating condition specifically.

It's not all doom and gloom. We spoke with our Proposals Manager, Jessica, last year about her daughter's rare condition and how they had coped during COVID-19. Jessica's daughter, Amelia, received a diagnosis for her rare autoimmune condition in 2019. Jessica commented, "although COVID-19 has produced new obstacles for Amelia we are fortunate that she has been safely thriving at home. She has been able to continue checking in with specialists through mainly telehealth appointments." Interestingly, Jessica also spoke about how virtual learning had actually benefitted Amelia and helped her engage with her classmates on a more level playing field.

Jessica and Amelia's telemedicine experiences highlight another option for rare disease research. It's well documented that patients are often vast distances away from their sites, due to the nature of rare populations. During COVID-19, telemedicine has offered a lifeline to medicine, but why should it stop there? Telemedicine could be used alongside a mobile research nurse – this would allow the site to "see" their patient whilst a research nurse could perform any procedures required by the study, reducing the burden on the patients but also keeping them safe.

In conclusion, rare disease patients and their families are some of the most committed and wonderful people within the clinical trial community. It seems to me that it is time research was catered to them and their needs; after all, without their commitment, no study in these smaller populations would get started. I am hopeful the pandemic has taught us the importance of patient-centricity within research and demonstrated how technology and clinicians can start to deliver new solutions within the rare community.

## REFERENCES

1. rarediseaseday.org, "meet the community".
2. <https://www.centerwatch.com/articles/24543-recruitment-rates-rising-but-retention-rates-fall-according-to-new-study>

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