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Interview with Marshall L. Summar: MAPPs in the Rare Disease Space

March 21st – by Alison Kilian

When it comes to rare diseases, it's important to find the fastest path and look beyond traditional trial models to meet patients' needs, says Marshall L. Summar, MD, co-chair of the rare disease advisory panel of Patient-Centered Outcomes Research Institute (PCORI) and the Scientific Advisory Committee of the National

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Organization for Rare Diseases (NORD). He is one of the world's leading authorities on accelerated pathways in orphan diseases and the uses of Real World Evidence in pediatric clinical research.

Patients in the rare disease space face unique challenges when it comes to clinical trials and the need to develop new medicines. "In the rare disease field, there really are no two patients alike," says Dr Marshall L. Summar, noting the difficulty this poses in developing appropriate treatments. When it comes to rare diseases, perhaps more than any other area, there is a pressing need to rethink the traditional approach of randomised clinical trials – and placebo control groups that can leave some patients hanging.

The traditional approval model of the US FDA (Food and Drug Administration) has been to wait for an overwhelming amount of evidence to be available in order to approve a drug, "and not as much after market approval". "In rare diseases, it is a challenge to have enough patients to amass that body of evidentiary proof on the front end in a timely fashion," explains Summar, who serves as the Division Chief of Genetics and Metabolism at Children's National Medical Center

Summar sees traditional models of medicines development as evolving in the future. "I think that eventually they will have to come up with a new pathway," he says. "Particularly for the ultra-orphans, there's no way to carry out a traditional models."

Greater Openness to New Models

For many in the rare disease community, a traditional randomised clinical trial is simply not a good option. "A lot of families today are not willing to enroll in a (traditional) trial, unless there is a certainty or a very good chance that they are going to be in the treatment arm." Given the uncertainty of these traditional routes – risk acceptance in rare disease populations is quite high, says Summar.

A Bayesian model such as MAPPs, he says, would be more agreeable. In comparison to traditional RCTs, "With a Bayesian model, you actually change the likelihood as the study progresses as to whether you end up in the placebo or the treatment group – and if the treatment is working, then you can get migrated over."

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Engaging the Rare Disease Community

There is no doubt that the rare disease community stands to benefit from exploring non-traditional development models like MAPPs. For Summar, the patients are absolutely ready: Compared to regular clinical trials, he characterizes clinical trials participation in the rare disease community as "a whole different scenario " with enrollment rates running high.

Summar also notes the greater flexibility of regulators when it comes to rare diseases: "The FDA has shown real willingness to have conversations with people wanting to do rare disease trials." This is not the case in traditional trials, where conversations and processes are more scripted.

For pharmaceutical companies looking to get involved in the rare disease space, Summar advises investing time in the community *before* the trial starts – with patients, care-givers, and advocacy groups. "First off, you'll learn a lot about how to design the trial – because they'll tell you a lot about the clinical state that you need to know before you do it. Secondly, it requires a very personal relationship to do a drug trial in these groups. You can't just parachute in with a CRO who doesn't know anybody and set it up."

About Marshall L. Summar

About NORD