

## CASE STUDY

# Enrolling a global natural history study: How to find 200 rare patients for a global natural history study

## Study synopsis

A sponsor selected Precision for Medicine to manage a global natural history study in a rare, X-linked kidney disorder. The study had been recommended by the FDA because there was extremely limited published data on this rare disease—the goal was to characterize renal function and decline in patients with the disease prior to initiating a phase 2/3 clinical study. The study, to be conducted in the United States, Canada, Australia and in Europe, would follow 200+ patients for approximately 2 years.

## Challenges

Enrollment was by far the greatest study challenge:

- The study targets a rare disease with a prevalence of 1 in 5,000
- A natural history study offers no therapeutic benefit for participants
- The target population with the most rapid kidney decline were young males, who had life commitments such as school and work that left little time for participation in a trial; many would have to travel to a clinic every 3 to 6 months
- A secondary target population, mothers of young boys with the disease, was more motivated to enroll, hoping their contribution might help lead to a cure; however, many had less aggressive disease progression, and so were not eligible to participate in the study

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**A natural history study, offering no therapeutic benefit for participants with a disease with a prevalence of 1 in 5,000, poses a distinct enrollment challenge**

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## Solutions

Precision took a multi-tiered approach to recruitment, enrollment, and retention, addressing each of the main challenges. First, we promoted the study through multiple channels where eligible patients might be able to learn about the study.

- Partnered with advocacy groups and rare disease organizations, making their members aware of the study through their social media, newsletters, events, and websites
- Launched a study-specific website and drove traffic to it through Google ads, WebMD, Facebook, Twitter, Instagram, and Pinterest
- Partnered with organizations that maintain a patient registry, informing their appropriate patients about the study via websites, email, mailings, and phone calls
- Hosted events and meetings at kidney conferences to help promote enrollment through potential referring physicians.

Once we found the patients, we deliberately eliminated hurdles to trial participation.

- Reduced patient travel burden by providing home healthcare for visits that did not need to be conducted in the clinic
- Reduced the financial burden for patients and caregivers by offering travel assistance and reimbursing out-of-pocket costs for travel, room, and board
- Fostered a strong connection with study site staff to encourage special attention for every single patient, their caregivers, and their personal situation, ensuring all their needs were met whenever possible to boost retention

## Results

Given that the disease is genetic, many patients had other family members who also qualified for the study. Because their study experience was so positive, they were willing to promote the study to others in their community. This magnifying effect, combined with ongoing recruitment efforts, resulted in a sufficiently robust study enrollment to generate the data needed to design a phase 2/3 study in the target patient population.

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For more information about our clinical trial solutions, please contact us at [info@precisionformedicine.com](mailto:info@precisionformedicine.com), or visit [precisionformedicine.com](http://precisionformedicine.com)

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