



AUGUST 2021

Choose Your Own Adventure

A unique MR approach to inform rare disease education

The challenge

One of the common challenges orphan drug makers face is how to improve the diagnostic process. In a landscape where every patient counts, ensuring an optimal diagnostic process is critical. However, rare diseases can present in complex, multisystemic ways, often leading patients down multiple incorrect paths before receiving a correct diagnosis - if they ever get diagnosed at all.

As clients develop strategies to improve this process, they must determine the optimal targets for investment in

educating physicians about their rare disease. Market research can help identify which specialties patients see during their diagnostic journey and which specialties could be more successful diagnosing patients if they were better educated.
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The market research solution

Many brands start market research programs with patient journey research, and this is an excellent foundation from which to select potential specialties of interest. Patient journeys reveal not just who ultimately diagnosed the disease, but who else the patient saw along the way. Once we understand which specialties are involved, we can then turn to market research with physicians in these specialties to evaluate them as targets.

An optimal target for a campaign will:

- 1. HAVE THE OPPORTUNITY**
to identify patients
- 2. BE WILLING**
to put in the effort
- 3. NEED ASSISTANCE**
to do so more effectively

Bringing real-world practice to an online survey

How do we use market research to determine how often physicians are missing, and misdiagnosing, patients with a rare disease? It's tricky, because we can't simply ask physicians how many cases they missed / misdiagnosed, especially if we're evaluating a disease with which they are unfamiliar. It doesn't help us better understand the diagnostic pathway if the physician doesn't believe they have ever seen a case of disease X in their practice.

What we can do is assess our three key criteria using blinded patient case studies.

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This allows us to simulate observation of the physician's diagnostic process. We've come to refer to this approach as "Choose your own adventure" research, because we follow physicians down a diagnostic path and provide feedback in response to decisions they make.

The first step is writing an appropriate case study. We want the case to be an accurate reflection of the information a physician might actually have when evaluating a patient who has the rare disease in question. We don't want to give away the case by making it too obvious (e.g., stating a relative had the disease of interest), nor do we want to write something too high level that will make the case impossible to decipher. Importantly, we have methods to account for multiple scenarios and the fact that physicians work through diagnostic processes in stages. We design cases that successively add information, and can dynamically update based on a physician's responses throughout the case (e.g., if a physician says they would order a specific test, we give them results).

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Once finalized, we present the case study to physicians and ask a series of questions to evaluate physicians on our three criteria.

CRITERION 1

Are physicians seeing patients who could have the rare disease of interest?

We assess this two ways. First, after showing the blinded case, we ask: how many patients have physicians seen who are similar to the case? Depending on how specific the case is and diseases in the differential, we can expect a range of responses to this question. Second, after revealing the disease of interest and providing basic education about it, we ask physicians if they would screen any of their current patients for this condition. This gives us a concrete estimate of the immediate impact education could have.

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CRITERION 2

Are there challenges with effective diagnosis?

This second criterion is important because if patients are already being effectively diagnosed, then there is no need for education. However, if (as we often find) physicians don't recognize the symptoms of the disease, are unaware it should be in the differential, or don't shepherd patients to more appropriate specialists, then education may be necessary. Using our blinded case study, we ask a few questions about what physicians would do if this

Specifically we ask:

- **What conditions could this patient have?**
We collect open-ended responses to limit bias identify patients
- **What would you do in this scenario?**
Refer the patient? If so, to what specialty? Run tests? If so, which ones?

patient came into their practice today. In our analysis, we determine whether physicians do think something that suggests they would reach the diagnosis of interest. We might classify a physician as likely to reach the diagnosis if they:

- **DIRECTLY SUSPECT** the disease,
- **REFER TO A SPECIALIST** already determined to be capable of diagnosing, or
- **RUN DIAGNOSTICS** that would reveal the disease of interest.

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This data helps us quantify how successful physicians currently are at diagnosing the disease of interest. We also learn about patient flow and whether patients may be retained vs. referred – another useful consideration for target prioritization.

CRITERION 3
Is the specialty of interest willing to be involved?

To answer this last question, we assess physicians' interest and willingness to be involved in diagnosis both before and after providing brief education about the disease. The pre-/post- comparison can also be used to simulate how physicians might change their behavior after being

exposed to an educational campaign. These insights are important not only for determining whether an educational campaign makes sense, but also to inform what an appropriate call to action may be for the campaign.

Improving the Diagnostic Process

By answering each of the three key questions, we understand the potential opportunity for improving the diagnostic process within the specialties of interest. This knowledge helps direct the significant investment decisions associated with launching new campaigns – and ultimately results in improved patient care.

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Fulcrum Research Group specializes in rare disease and healthcare-related market research with a focus on creativity, teamwork, and partnership. We opened our doors in 2010 in Waltham, MA.

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