

# From KOLs to Patient Advocates, Using Every Tool to Fight a Rare Disease

Finding patients for trials in very rare conditions is difficult under any circumstances, and in this case the odds were really stacked high against success.

The indication: a basal cell carcinoma known as Gorlin syndrome, a disease that manifests itself in large numbers of benign tumors that grow on various parts of the body. The sponsor: a very small, virtual company consisting of a handful of contractors pursuing success in the form of a topical gel, which had only ever been tried as an oral drug in the past. This had demonstrated several undesirable side effects for patients as an oral medication, and the only other alternative available was to undergo numerous surgeries to physically remove the tumors.

Our recruiting target was 18 patients, but even that was a challenge for a condition that afflicts just one in 31,000 people. It required making full use of what few resources were available, including key opinion leaders (KOLs) at the two trial sites, both in the United Kingdom, chosen because of their familiarity with Gorlin syndrome and their ability to spread the word of the study amongst their professional peers. One of these KOLs even agreed to present a summary of the study at a British Association of Dermatology conference.

## Engaging patient advocates

We engaged the Gorlin Syndrome Group, a patient advocacy group based in the U.K., which publicized the study on its website, [gorlingroup.org](http://gorlingroup.org), and circulated an advertisement to its member mailing list. We created a patient invitation letter that went to every prospective patient identified through these networking efforts in order to introduce them to the study at a high level.

The work paid off. We located our 18 patients ahead of schedule and completed the 26-week trial early, ultimately providing the final deliverable – the electronic trial master file (eTMF) – ahead of the contracted schedule date.

The study demonstrated the efficacy of the topical treatment and showed a very good safety profile. In late 2017, the Food and Drug Administration granted the drug Breakthrough Therapy and Orphan Drug designation, and the sponsor is now preparing for a Phase III trial.

# RARE DISEASE



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# Finding 18 Patients for a Six-Month Study Was Harder Than You Might Think

## Study Description:

Phase IIA study of a topical treatment for Gorlin syndrome, a basal cell carcinoma that causes large numbers of benign tumors

## Therapeutic Area:

Dermatology

## Geographic Scope:

Two sites in the United Kingdom

## Patient Population:

18 patients

## Length of Enrollment Period:

6 months

## Outcome:

Phase IIA trial completed successfully; planning for Phase III trial now underway



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