

Horizon Therapeutics Effectively Engages Patient Organizations to Improve Development of First Approved Medication for Thyroid Eye Disease

Horizon Therapeutics Applies CTTI's Patient Group Engagement Recommendations

SUMMARY

Horizon Therapeutics inherited teprotumumab (now marketed as TEPEZZA) as part of a 2017 acquisition. Previous research showed the medication had promise to be the first U.S. Food and Drug Administration (FDA)-approved treatment for Thyroid Eye Disease (TED), a rare condition that causes bulging eyes, pain, double vision and, sometimes, blindness -- but first, Horizon had to complete a phase 3 confirmatory study. Here is how Horizon used CTTI's Patient Group Engagement (PGE) recommendations to ensure feedback from and collaboration with the patient community during the development of TEPEZZA, the first-ever treatment for TED.

GOAL(S)

There are currently more than 7,000 rare diseases identified, and only five percent have approved treatments. With the acquisition of teprotumumab (now marketed as TEPEZZA), Horizon Therapeutics saw an opportunity to bring patients the first FDA-approved treatment for one of those rare diseases: Thyroid Eye Disease, or TED. Signs and symptoms of TED include eye bulging, eye pain, double vision, light sensitivity or difficulty closing the eye. Although TED impacts relatively few individuals, it can be incapacitating. Symptoms can lead to the progressive inability of people with TED to perform important daily activities, such as reading, driving or working. TEPEZZA had performed well in a previous phase 2 study published in the *New England Journal of Medicine*, but Horizon needed to complete a confirmatory study to bring it to market.

CHALLENGES

With no FDA-approved treatment, people afflicted with TED typically had to wait for the disease to become inactive (six months to two years) before pursuing surgical options to restore appearance and function. Even then, many individuals needed further surgery to realign the eyes. For these patients, an approved biologic could be a life-changer, saving them from complex surgeries and significantly elevating their quality of life. Horizon needed to conduct its phase 3 confirmatory trial in a way that would fast track the approved treatment to TED patients, who were eagerly anticipating an alternative to watching and waiting for surgery.

SOLUTION(S)

Horizon's chief medical officer had been involved for several years with CTTI's patient engagement efforts, exploring best practices that led to CTTI's PGE recommendations for engaging with patients to optimize clinical trial design and execution. On his recommendation, Horizon reached out to the Graves Disease and Thyroid Foundation (GDATF), a patient group that provides education and support to people and their families struggling with thyroid disorders. Horizon hoped that by engaging with the patients who were experiencing TED, they could help to ensure awareness of the Phase 3 clinical trial so that potential study participants knew how to find information about the study. The company also sought to listen and learn from people who have lived with the disease, as understanding these challenges would help contribute to the development process.

TAKING ACTION

Horizon asked the GDATF for an opportunity to host a focus group during GDATF's 2018 Patient and Family Conference. After consultation with the GDATF Board of Directors, the GDATF agreed to move forward. (The Board recommended that the focus group opportunity be made available to other potential sponsors in the pharmaceutical space, but the other organizations either declined or did not respond.) The focus group drew about 12 conference attendees. Several of those focus group participants expressed interest in having further discussions with Horizon, and in response, Horizon launched a TED council made up of Horizon employees and about 20 people who were either currently or previously experiencing TED. The council convened every two months and talked about the TED treatment experience, such as what it is like to have infusions, and what works and doesn't work in terms of education and information. Using CTTI's PGE recommendations as a guide, the council consciously and proactively thought through the patient experience. From Horizon's perspective, this was key in helping to ensure that engagement and educational efforts with the TED community were conducted with the first-hand perspective of people living with TED. "They were very open and receptive to our input," said GDATF's founder, who participated in the TED council to provide her own experience with TED. "For example, in one meeting the study team was developing a slide on how TED patients could tape their eyes shut at night, and I told them that most TED patients tape their eyes differently. Horizon was so eager to hear more. They updated their educational materials based on this feedback, which made our patient experience feel valued."

IMPACT

The patient community was able to provide input for Horizon on the experience of living with TED, which helped shape the company's efforts to raise awareness of the Phase 3 clinical trial. In addition, GDATF informed its community members of the clinical trial information available on clinicaltrials.gov. The original phase 2 study for TEPEZZA took several years to complete. Horizon was aiming to get the phase 3 confirmatory study enrolled in one year. However, Horizon was able to achieve enrollment in just nine months. What's more, over 90 percent of patients enrolled in the study stayed through completion -- the dropout rate was incredibly low, and results for the study were highly positive. The FDA approved TEPEZZA in January 2020 as the first treatment for TED following full advisory committee support in December 2019. In addition to giving the biologic Fast Track and Breakthrough Therapy Designation, the FDA granted TEPEZZA's application priority review with completion in 4.5 months instead of six. TEPEZZA also received Orphan Drug designation, which provides incentives, like research grants or tax credits, to assist and encourage the development of drugs for rare diseases or conditions.


ADVICE

Horizon approaches all its rare disease research with the understanding that patient input is key, and the patient could be any one of us. Their chief medical officer calls it "uniting a village of voices" across the research spectrum (including academia, regulators, patients, and contract research organizations). Horizon seeks to engage its disease communities throughout the lifecycle of its medicines, from development to commercialization. The company notes that its patient relationships are not transactional, one-and-done study collaborations. For example, when an FDA Advisory Committee meeting took place in December 2019, members of the TED council gave testimony on the patient experience with TED. "There are so many benefits to including patient input in the development cycle," said GDATF's founder. "This was a unique advocacy opportunity, and we are so happy that our TED community now has a non-surgical option to consider."

ORGANIZATION

Horizon Therapeutics [Acquired 2023]

CONTACT

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ORGANIZATION TYPE

Industry

IMPLEMENTATION DATE

2018

TOPIC

Patient Engagement

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CTTI RESOURCES

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[Teprotumumab for the Treatment of Active Thyroid Eye Disease](#)