PCM TRIALS
QUALITY MOBILE RESEARCH

The Largest Parkinson's Disease Study Ever Attempted is Being Made Possible Only with Mobile Research Nurses

ABOUT THE STUDY

The home-based Trial of Parkinson's and Zoledronic acid (TOPAZ) is investigating whether a drug used to treat osteoporosis will reduce bone fractures in Parkinson's disease patients.

Due to the limited mobility of many of the participants and the need to enroll so many patients, the only way this trial is possible is using decentralized clinical trial methods including mobile research nurses, which are provided by PCM Trials.

This case study, based on a presentation at Outsourcing in Clinical Trials (OCT) East Coast by Steven R. Cummings, MD, Executive Director, San Francisco Coordinating Center, illustrates the many advantages of home-based clinical trials including:

- Increased convenience for patients, including those with limited mobility
- Larger number of participants than possible with a clinic-based trial
- Potential for increased diversity in clinical trial participation
- Reduced costs

Largest Clinical Trial Ever Attempted for Parkinson's Disease by 10-Fold

Estimated 80% Cost Savings Per Participant Compared with a Similar Trial Using Clinical Sites

PCM Trials Nurses Trained in Study Protocol in 40 States and Counting

Aiming to Enroll 3,500 Participants Over Age 60

SITUATION

Because Parkinson's disease is a progressive brain disorder that leads to shaking and stiffness, eventually causing difficulty with walking, balance and coordination, patients are at very high risk of fractures from multiple and sometimes severe falls that can lead to permanent disability and even death. Researchers are studying whether zoledronic acid, approved by the FDA to treat osteoporosis by increasing bone strength, will reduce fractures in people with Parkinson's disease.

The Trial of Parkinson's and Zoledronic acid (TOPAZ)[1] is seeking to enroll 3,500 participants over the age of 60, making it the largest double-blind, placebo-controlled, randomized clinical trial ever attempted in people with parkinsonism.

Anecdotally, only a very small proportion of people with parkinsonism receive treatment to reduce fracture risk. Barriers to seeking or receiving treatment include:

- a lack of evidence that medical treatments will reduce fracture risk in this population;
- the conventional approach to test for bone mineral density (BMD) and have follow-up medical visits to interpret, prescribe and assess response to treatment is unfamiliar to the neurologists who typically treat Parkinson's patients; and
- the notably poor compliance with oral osteoporosis treatments may be even worse for older people with parkinsonism who are already burdened by taking several other medications.

TOPAZ was designed to overcome barriers to treatment by allowing people to enroll without the need for a doctor's referral or BMD testing, treating participants with an intravenous infusion vs. an oral medication and, importantly, making the trial conveniently home-based.

The need to enroll such a large number of participants, many of whom have mobility and/or cognitive challenges, would make a traditional site-based study impossible. Even if the required number of prospective participants could be found living within in a reasonable proximity to a clinical site, travel to and from the site would be very difficult if not impossible for many participants. It could also be time-consuming and expensive for the participant to get to the site and would put a burden not only on the patient, but also their caregivers.

The study recruitment began October 1, 2020, and is expected to be completed within three years.

APPROACH

Based on previous conversations with PCM Trials, researchers knew that a trial that would have been impossible to even imagine if it were site-based would be feasible using mobile research nurses. By involving PCM Trials in planning from the very beginning, they were able to design a study that could recruit a large number of participants from across a wide geography and develop a multi-step protocol that could be done entirely from participants' homes.

PCM Trials manages the study drug, including storage, blinding, randomization, and delivery to its Certified Mobile Research Nurses who have been trained on the study protocol. Participants receive a supply of vitamin D supplements and are instructed to take the medication for a period in advance of the nurse's home visit to prevent the very low risk of hypocalcemia after the infusion. At the home visit, the participants are given one dose of the study drug intravenously. This treatment has been shown to prevent fractures in people with osteoporosis, with the preventive effects persisting for two years.

During the home visit, the nurse:

- obtains a "wet ink" informed consent signature (which supplements the online signature the participant would have provided when they enrolled online),
- confirms that the patient has complied with the pre-infusion vitamin D regimen,
- examines the mouth to ensure that there are no lesions that would predispose the patient to a rare side effect of the medication. If the oral exam is questionable, the nurse uses a secure smart phone protocol to send an image of the mouth to the study physician who immediately reviews it for eligibility,
- takes a blood sample to obtain a rapid point-of-care measurement of renal function,
- administers the I.V. study drug or placebo,
- gives the patient acetaminophen to prevent a potential reaction to the infusion and instructs the patient on how to take the medication over the next couple of days to prevent side effects.

In terms of communicating with the investigators, the PCM mobile research nurses:

- promptly communicate with the Coordinating Center through every step in the process as new patients are enrolled and home visits conducted,
- refer patient questions to the TOPAZ help line,
- track the process from the time of enrollment to the scheduling and completion of visits,
- participate in TOPAZ Steering Committee calls every two weeks.

RESULTS

Because TOPAZ is a home-based trial, researchers can meet their goals of recruiting an unusually large number of participants from across the U.S., without the geographic constraints that would come with a site-based trial. Being able to recruit from a broad geographic area is enabling the study to include participants who are more representative of "real world" patients. And the fact that the study is patient-centric and convenient, which is especially important to a population of older patients with limited mobility, is also making it easier to recruit participants. The trial is ahead of its recruitment goals.

In addition, having a home-based study focuses researchers on just those activities outlined in the study's protocol. This, along with the fact that there is no overhead or staff costs of a clinical site, have resulted in an estimated cost savings of 80% per participant compared with a similar trial conducted in a clinical site.

This case study is based on a presentation delivered at Outsourcing in Clinical Trials East Coast[2] by Steven R. Cummings, MD, The San Francisco Coordinating Center. The San Francisco Coordinating Center is a non-profit academic research organization with more than 20 years of experience conducting multicenter studies and clinical trials.

To watch the presentation, "Clinical Trials from Home, The Role of Research Nurses" visit www.pcmtrials.com/topazcasestudy.

To learn more about how Decentralized Clinical Trial methods can help your trial, contact PCM Trials at 866.631.9985, info@PCMTrials.com, visit www.PCMTrials.com, or submit an RFP today.

1. Tanner, Caroline M., et al. "The TOPAZ study: a home-based trial of zoledronic acid to prevent fractures in neurodegenerative parkinsonism." NPJ Parkinson's disease 7.1 (2021): 1-6.

2. Cummings, Steven R. (2021, May 25-26). Clinical Trials from Home: The Role of Research Nurses [Conference presentation]. Outsourcing in Clinical Trials East Coast 2021, Virtual Event.

"Home visits by research nurses made it possible for us to imagine TOPAZ. It was possible to imagine doing something that was very large and that could be done conveniently for older patients with a disabling disease. It became possible to imagine U.S.-wide recruitment. From previous conversations with PCM Trials, we knew that it was feasible to do all of this and a multi-step protocol from home.

I've learned that it's much better to plan home-based trials in advance, from the very beginning, than to try to retrofit protocols. I involved PCM Trials in the very first planning discussions and we worked with them along the way at every step to develop the proposals and the protocols."

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