

wahlig

Von: PatientInfo [PatientInfo@ptcbio.com]
Gesendet: Mittwoch, 19. Dezember 2007 18:20
An: PatientInfo
Betreff: PTC124 Update from PTC Therapeutics

Dear Friends of PTC,

PTC Therapeutics would like to provide an update on the current status of our investigational drug, PTC124.

PTC124 targets a genetic change known as a nonsense mutation. A nonsense mutation causes a genetic disorder by interrupting the production of a critical protein. PTC124 may have the potential to treat genetic disorders in patients for whom a nonsense mutation is the basis for the disease, such as Duchenne muscular dystrophy (DMD) and cystic fibrosis (CF). PTC Therapeutics recently completed short-term clinical trials for PTC124 in DMD and CF; a summary of the results is available on our website (www.ptcbio.com).

PTC is initiating longer-term clinical studies of PTC124 in patients with nonsense-mutation-mediated DMD and CF. The main goals of these studies are to understand whether PTC124 can improve how patients feel and function and whether the drug can safely be given over periods of many months. These trials will be multi-center, randomized, double-blind, placebo-controlled studies. In addition, a DMD 2a extension study is being planned. Please visit our website (www.ptcbio.com) for additional information regarding the trials.

To be considered for these studies of PTC124, participants must have been gene-sequenced and know that a nonsense mutation is the basis for their DMD or CF. Patients with a genetic disorder who have not been genotyped should consider discussing gene-sequencing with their treating physician or genetic counselor. Knowing the genetic sequence of a disease-causing gene may prove useful in determining eligibility for clinical trials involving certain types of experimental therapies. Facilities that perform gene-sequencing can be located through the Gene Tests website (www.genetests.org); click "Laboratory Directory" to search for the locations of gene-sequencing facilities.

In addition, study participants for the DMD trial must be at least 5 years of age and be able to walk at least 75 meters (80 yards) unassisted in a 6-minute time period. Other inclusion criteria will be explained prior to trial enrollment. PTC plans to initiate the DMD trial in the next few months, followed by the CF trial. If the planned studies of PTC124 in DMD and CF yield positive results, we hope to conduct clinical studies in additional nonsense-mutation-mediated genetic disorders in the future.

To stay informed about PTC124, clinical trials, additional information about genetic testing, and other news about PTC Therapeutics, please join our mailing list by visiting our website (www.ptcbio.com/contact_form).

Best Regards,

PTC Therapeutics

If you would like to be removed from this mailing list, please email info@ptcbio.com and write "remove" in the subject field.

THIS ELECTRONIC MAIL MESSAGE AND ANY ATTACHMENTS ARE CONFIDENTIAL AND MAY CONTAIN PRIVILEGED INFORMATION INTENDED ONLY FOR USE OF THE INDIVIDUAL OR INDIVIDUALS NAMED ABOVE. If you are not an intended recipient, you are hereby notified that any dissemination, distribution, use, disclosure, printing or copying of this communication is strictly prohibited. If you have received this communication in error, please reply to the sender, or help@PTCBio.com, to notify us of the error and delete the original message and any attachments. The views or opinions presented in this message are solely those of the author and do not necessarily represent those of PTC Therapeutics. Thank you.