“International Clinical Program Update”

Niemann-Pick – UK Conference
September 18 - 20, 2015, Wyboston Lakes, UK

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Scientific Advisory Board

Sharon H. Hrynkow, PhD
Co-Chair

Caroline Hastings, M.D./Pediatric Oncology/Hempel-Hastings IND/Shares Protocol Globally

James E.K. Hildreth, M.D., Ph.D./President, Meharry Medical College

Benny Liu, M.D./Gastroenterologist/Seminal studies on NPC in animals

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Trappsol® Cyclo™ Development Milestones (1)

- 2008 – Hempel family contacts CTD for assistance in obtaining cyclodextrin
- 2009 – CTD’s Trappsol® Cyclo™ provided to Caroline Hastings for treatment of Hempel twins
- 2010 – CTD’s Trappsol® Cyclo™ provided to Pessoa family in Brazil
- 2011 – 2015 Compassionate use grows
- 2012 – Distribution agreement for South America established with Uno Healthcare (for compassionate use program)
- 2012 – Second generation Trappsol® Cyclo™ begins distribution
- 2013 – Distribution agreement for Europe established with Medical Need Europe (MNE) (for compassionate use program)

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• 2014 – CTD launches program to develop International Clinical Program
  • Manufacturing validation of Trappsol® Cyclo™ completed

• 2015 – Orphan Drug Designations transferred to CTD via its partner MNE from Sue French (for UK & Europe) and Caroline Hastings (US)
  • Type II Drug Master File accepted by U.S. FDA
  • **August 2015:** CTD announces plans for Intravenous Trappsol® Cyclo™ clinical trial and upcoming meeting with European health authority to discuss initiation of clinical program.

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Drug Master File; On file with FDA

A Drug Master File (DMF) is a submission to the FDA of information, including the Chemistry, Manufacturing and Controls (CMC) of a drug product, or a component of a drug product, to enable the FDA to review this information in support of a third party’s submission.
International Clinical Program

• Goal: Gaining market approval for use of Trappsol® Cyclo® in the treatment of NPC
• Initial steps: 2013 – 2015
• Established an expert team to develop/conduct the clinical program
  • Dr. Sharon Hrynkow, first as consultant, now as Senior VP for Medical Affairs
    • Accenture Accelerate R & D Services for Life Sciences
    • Boyd Consultants -- Alan Boyd
    • Aptus Clinical
    • KJC Statistics
    • Prism Ideas
• Why an Intravenous trial?
• NPC is a systemic disease
• Cyclodextrins provided to mice in a systemic manner lead to reduced total cholesterol, decrease in liver size, increase in longevity

Systemic Weekly Administration of Cyclo™
Weekly Cyclo™ Prolongs Lifespan

- Untreated (n=76)
- CYCLO once at 7 days (n=32)
- CYCLO given weekly (n=20)

Survival (%) vs. Age at Death (days)

P < 0.0001
• Why an Intravenous trial?
• Anecdotal clinical data available for IV administration for up to five years
• Published reports on IV administration leading to stabilization of symptomology or improvements, including in CNS (“wakefulness”)
• IV less invasive than some other routes of administration
• IV easier for families and physicians to manage
• IV does not require sedation
• When will the trial start?

Expected enrollment first half of 2016.
• Information sharing for Scientists, Parents, and Physicians

• Brings the voices of families and physicians into the clinical program development process

• Operational throughout, and continuing after, CTD’s International Clinical Program

• Learning, sharing – CTD listens to those most affected

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Family and Physician Listening Circle

Sue French
Co-Chair – UK/Parent

Caroline Hastings, M.D.
Co-Chair – US/Physician

David French, – UK/Parent

Robin Lachmann, M.D.
UK/Physician

Begoña Arias, M.D.
Spain/Physician

Camilo Vieira, M.D. – Brazil/Physician
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Roberto and Renata Pessoa
Brazil/Parents
Join the Listening Circle

• We cordially invite you to participate in our Family and Physicians Listening Circle

• CONTACT INFORMATION
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Clinical Protocol Collection

• Purpose: Collect and assemble a library of adaptations of the original Hastings/Hempel IND protocol to build knowledge on delivery of Trappsol® Cyclo™ and other cyclodextrins.

  • Provides us with up to date information on current uses
  • Informs our clinical program

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CTD’s cyclodextrin (Trappsol® Cyclo™) in use in 20 patients right now, and it has been in use in some for more than 5 years.

Trappsol® Cyclo™ has a consistent manufacturing process, a DMF as an Active Pharmaceutical Ingredient and has proven stability.

Anecdotal data suggest that an IV trial will be important for NPC Patients and their Families.

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Summary (2)

• We are working to launch our IV trial in the first part of 2016

• Compassionate use programs will continue in parallel with clinical programs

• We look forward to working with the whole NPC community to improve the lives of NPC patients globally.
For more information, please contact us at:

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