

First International Chordoma Research Workshop

May 3-4, 2007

Bethesda, Maryland

Summary

The First International Chordoma Research Workshop is a Great Success!

On May 3-4, 2007 the Chordoma Foundation co-hosted the First International Chordoma Research Workshop along with several NIH institutes including the National Human Genome Research Institute (NHGRI), Office of Rare Diseases (ORD) the National Cancer Institute (NCI), and the National Institute for Neurological Disorders and Stroke (NINDS). Because chordoma does not fit neatly within the domain of any one institute, it was especially valuable to have the involvement of multiple NIH representatives. The Chordoma Foundation conceived the workshop in December 2006 and worked rapidly with a dedicated organizing committee comprised of scientists from NHGRI, ORD, NCI, NINDS, Duke, and Harvard. Ultimately, the workshop was made possible by the support of Drs. Francis Collins and Alan Guttmacher, and their staff at NHGRI who graciously assisted the Chordoma Foundation in coordinating this event.

The First International Chordoma Research Workshop was conceived to promote collaborations among various medical disciplines and to bring new researchers into the chordoma research community. The workshop brought together a multidisciplinary group of researchers and clinicians from around the world with the charge of formulating a roadmap for the future of chordoma research. Participants at this landmark event included both scientists deeply involved in chordoma research, and experts in related fields. Thanks to everyone's active participation, the workshop was filled with vigorous and fruitful discussions. The excitement in the room was palpable as former strangers united as a team in pursuit of a common goal. Together we laid the foundation for the future of chordoma research and formally initiated a coordinated international effort to cure this devastating disease.

The Chordoma Foundation will serve as a hub for organizing and directing the multidisciplinary and multi-institutional collaborations that emerge from the conference. We will do everything possible to make life easier for researchers and lower the activation energy necessary for initiating and sustaining collaborative projects. We hope to catalyze these projects by providing easy access to biological material, connecting researchers, providing logistical support, and acting as a liaison between patients and researchers.

Participants

Workshop participants represented a wide variety of specialties including tumor biologists, geneticists, surgeons, pathologists, oncologists, radiologists, embryologists, pharmacologists, and fellow rare disease advocates. Several major academic centers were represented including, Massachusetts General Hospital, Dana Farber, Memorial Sloan Kettering, Johns Hopkins, Duke, MD Anderson, Children's National Medical Center, Roswell Park, St.Lukes-Roosevelt, University of Arkansas, and the University of Pittsburgh. Additionally, international researchers

came from Japan, England, Italy, and Canada. The diverse perspectives of these fifty-three participants allowed for significant cross-fertilization of ideas and identification of numerous relevant research avenues. Though most participants had never met prior to the workshop, there was much collegiality during the two days and many left with a sense of camaraderie, pledging to collaborate on new projects.

Agenda

The one-and-a-half day meeting included both plenary and small working group sessions (see agenda). The group discussed the current state of the science surrounding the tumor biology and genetics of chordoma, as well as the path to treatment development. Interestingly, many of the presentations shared preliminary data generated from research collaborations initiated by the Chordoma Foundation in the four months leading up to the workshop. This new data helped inform the debate and generated testable hypotheses that contributed to the formation of a research action plan.

Breakout groups focused on three areas: 1) Mechanisms of Disease, 2) Experimental Therapeutics, and 3) Future Clinical Management. Each group set goals for advancing the field and then identified resources and specific actions needed to accomplish those goals. Breakout group moderators compiled this information and presented it to the larger group for discussion. What emerged was broad consensus on a comprehensive plan for conducting basic science, translational, and clinical research in parallel. Finally, Dr. Francis Collins wrapped the meeting up by distilling the group reports into a prioritized action plan, which will serve as the roadmap for a coordinated international research initiative.

The meeting was closed with touching remarks from several patients and parents. They expressed their gratitude to the researchers and drove home the importance of rapidly finding a cure to help improve the lives of the many people who deal with chordoma on a daily basis.

Major Outcomes

- 1) A comprehensive action plan to guide research
- 2) Numerous new relationships formed between researchers and clinicians
- 3) Broadened the chordoma research community: generated new interest and increased awareness among top researchers in numerous fields
- 4) New projects, collaborations, and pledges of support
- 5) Multidisciplinary research teams formed at institutions in the US, Canada and Europe
- 6) Clarity regarding the needs of researchers and the role of the Chordoma Foundation in coordinating and supporting multiple research projects
- 7) Formation of several expert bodies: **Clinical Management Guidelines Committee, Clinical Trials Working Group, Chordoma Foundation Think Tank, Chordoma Foundation Scientific Advisory Board**

Next Steps

- 1) Convene Clinical Trials working group to prioritize research protocols and begin enrolling chordoma patients in clinical trials
- 2) Convene Chordoma Management Guidelines Committee to create consensus statements by the end of 2007
- 3) Create online communication network
- 4) Set up Chordoma Foundation BioBank, collect tissue, create tracking and surveillance system
- 5) Begin genome-wide tumor profiling

Members of the workshop organizing committee are in the process of compiling the workshop proceedings for publication. Additionally we are refining and formalizing a more comprehensive version of the strategic plan which will be posted on our website shortly.

This is only the beginning. As Dr. Collins said, “we’re turbocharged!” **The Second International Chordoma Research Workshop** is already scheduled for April 2008. Until then, we’ll work tirelessly to advance the field in hopes that one day soon a cure will be found.