

Third International Chordoma Research Workshop



Eighty-five physicians and scientists gathered in Bethesda, Maryland from March 17-19, 2011 to exchange the latest discoveries about chordoma and build partnerships with colleagues from across the world. Researchers came from thirty-one institutions in nine different countries, including – for the first time – four different pharmaceutical companies. At the workshop thirty-two speakers, including six Chordoma Foundation grant recipients, presented new, unpublished data spanning the gamut from epidemiology to genomics to clinical trials.

A number of significant developments were shared for the first time, including the following highlights:

Chordoma patients are surviving longer

Dr. Mary McMaster from the National Cancer Institute Genetics Epidemiology Branch re-analyzed nation-wide cancer registry data and found that the median survival of chordoma patients has increased from 7 years to 9 years.

Genomic analysis has revealed mutations that could be driving chordoma

Researchers from the Sanger Institute, the NIH Intramural Sequencing Center, the British Columbia Cancer Agency and Massachusetts General Hospital presented preliminary findings from analyzing the chordoma genome with a variety of technologies including: exome sequencing, paired-end rearrangement analysis, kinome sequencing, and transcriptome sequencing. Mutations were identified in a number of known cancer genes including PTEN, Akt, and PI3K which belong to a signaling pathway recently discovered to be highly active in chordomas. Work is ongoing to increase the number of samples sequenced and determine the functional significance of the mutations identified.

Brachyury is a promising therapeutic target

Several speakers presented data suggesting that the gene brachyury plays an important role in driving chordoma as well as other types of cancer. Dr. Adrienne Flanagan from the University College London and Dr. Wesley Hsu from Johns Hopkins University both found that knocking down brachyury causes chordoma cells to stop growing. Work is ongoing at both institutions to develop ways to knock down brachyury in chordoma tumors. Additionally, researchers at the National Cancer Institute are working on an immune therapy approach to selectively destroy cells that express brachyury.

New cell lines are in development

Researchers from Massachusetts General Hospital, Johns Hopkins University, the Medical University of Graz in Austria, and the University of Ulm in Germany all showed new chordoma cell lines at various stages of development. Each of these groups continue to work on developing new cell lines, and have pledged to contribute their cell lines to the Chordoma Foundation cell line panel once they are fully validated. Thus far, the Chordoma Foundation has distributed the two cell lines in its cell line panel to 33 different labs.

New mouse models of chordoma have been developed

Reseachers from Massachusetts General Hospital, Johns Hopkins Univeristy and the Istituto dei Tumori in Milan presented detailed characterization of five newly developed mouse xenograft models of chordoma (human chordoma tumors grown inside mice). These researchers and others have been trying unsuccessfully for years to get chordomas to grow in mice (until now only [one xenograft had been reported](#)), but have now learned methods that increase the odds of success.

Two clinical trials are nearly complete; three new clinical trials are set to open

The Sarcoma Alliance for Research through Collaboration (SARC) enrolled 34 chordoma patients on SARC009, a Phase II study of dasatinib. Dr. Scott Schuetze from the University of Michigan presented preliminary data showing that dasatinib induced disease stabilization in a number of patients - some for greater than two years.

Dr. Silvia Stacchiotti from the Istituto dei Tumor in Milan reported final results from a Phase II study of imatinib in 50 chordoma patients. Over sixty percent of patients treated with imatinib experienced a partial response or stable disease for at least six months. Dr. Stacchiotti also reported that her group has opened two new Phase II trials with targeted therapies for patients with advanced chordoma: one with imatinib plus mTOR inhibitor RAD001, and one with the EGFR/Her2 inhibitor, lapatinib.

Dr. Deric Park from the University of Virginia has opened a Phase I-b trial of imatinib in combination with the HDAC inhibitor LBH589 at the University of Virginia, the University of Michigan, and Massachusetts General Hospital. [Details about this trial.](#)

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March 20, 2011