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Donor Dollars at Work
Second Chordoma Community Conference
June 27, 2009
Outline

1. Research Roadmap
2. Resources needed
3. Model Systems Grants
4. Hear from funded researchers
   - Brian Harfe, PhD
   - Vijaya Ramesh, PhD
   - David Alcorta, PhD
   - Jean-Paul Wolinsky, MD
5. Drug screening project – Chris Austin, MD
6. Chordoma Genome Project
Our mission is to rapidly develop effective treatments, and ultimately a cure for chordoma, while improving the diagnosis, treatment and quality of life for chordoma patients.

How?
Strategy

By leading a coordinated, multidisciplinary research effort, and systematically breaking down the barriers that stand in the way of progress

» Communication & Collaboration
» Access to scientific resources
» Funding
Research Roadmap

Resources
Develop and share scientific resources needed to study the molecular biology of chordoma, and test new treatments

Molecular Discovery
Analyze chordoma using all available technologies and techniques to uncover its molecular underpinning and defining features

Target Identification
Integrate results from in-vitro, in-vivo, and in-silico research to identify the molecules and pathways that chordomas rely on for survival

Translation
Identify and develop therapies that selectively inhibit chordoma survival, and test these therapies, alone and in combinations, on in-vitro and in-vivo models

Clinical Research
Test the most promising therapies in clinical trials. Identify biomarkers predictive of response to inform molecular discovery and enable increasingly individualized therapy

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Scientific Resources

Model Systems
» Cell Lines
» Transgenic Animals
» Xenographs...

Clinical Data
» Treatment History
» Family History
» Tests & Images...

Biospecimens
» Tumor Tissue
» Blood
» DNA...

“Omic” Data
» Gene Sequence
» Gene Expression
» Protein Expression...
# Model Systems

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<th>Model</th>
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| Cell Lines     | • Live tumor cells grown perpetually in Petri dishes  
• Easiest of any model to grow and manipulate  
• Used for a wide range of experiments                                             |
| Xenographs     | • Human tumors implanted in mice  
• Thought to more closely mimic human tumors                                         |
| Transgenic Animals | • Animals genetically engineered to develop tumors that mimic specific human cancers  
• Valuable for understanding the underlying biology of human tumors                  |

- Each model has its own unique benefits and drawbacks
- Using all three models in combination gives scientists the most complete understanding of how the disease works and how to treat it.
Model Systems Grants Program

- Grant review process guided by American Cancer Society Grants Program Manager, Dr. Bill Phelps
- 10 applications from 6 countries
- Four peer reviewers from Duke, Harvard, Vanderbilt
- **6 Grants awarded**
  - 4 $25,000 model systems grants awarded
  - 1 co-funded grant with Liddy Shriver Sarcoma Initiative
  - Grant to support research scientist at Duke
Awarded Grants

Brian Harfe, PhD
University of Florida
» Transgenic Mouse

Michael Kelley, MD
Duke University
» Cell lines
» Drug Screening

Vijaya Ramesh, PhD
Massachusetts General Hospital
» Transgenic Mouse

Adrienne Flanagan, MD, PhD
University College London
» Cell lines

David Loeb, MD, PhD
Johns Hopkins University
» Xenographs

Elena Tamborini, PhD
Istituto di Tumori, Milan
» Xenographs