



## *From the Director: Technology Validation Takes Hold*

**By Joseph D. Fondacaro, PhD, Director,  
Office of Intellectual Property and  
Venture Development**

In this issue of our newsletter, we are pleased to announce the first awards of the Children's Technology Validation Fund (CTVF). The CTVF has been established to provide financial support for validation studies on promising technologies identified as commercial opportunities. Technology validation is an important step for academic research centers in order to attract the attention of licensees and investors to early stage discoveries.

Following the submission of an invention disclosure to our office, patentability and marketability of the

new invention are determined. If a patent application is submitted on the new technology, the inventors are asked to present a review of the technology, a validation study proposal and a budget to the Technology Validation Advisory Board (TVAB). If the TVAB advises that the technology is on a commercial track, the validation proposal can be submitted to a CTVF committee with a request for funding.

Validated technology significantly increases the likelihood of a commercial license for CCRF. While still a pilot project, it is anticipated that the CTVF will grow as a result of future licensing revenues being set aside for technology validation.

## *Cincinnati Children's Announces First Validation Fund Awards*

In the December 2003 newsletter, an announcement was made regarding an internal technology validation fund at Cincinnati Children's. This fund will allow the medical center to further develop its technologies, to make them more attractive for licensing - either as a startup or to an existing company.

Technology validation is a critical step in the commercialization strategy of the Office of Intellectual Property & Venture Development. "We need to move our technologies further along the development path," noted Joseph Fondacaro. "But we want to make sure we do so in an appropriate way, with the input of the commercialization experts."

An external review panel, comprised of four members of the Children's Technology Validation Advisory Board (TVAB), made recommendations that were adopted by Cincinnati Children's. Three technologies were selected to receive validation grants in the first quarter:

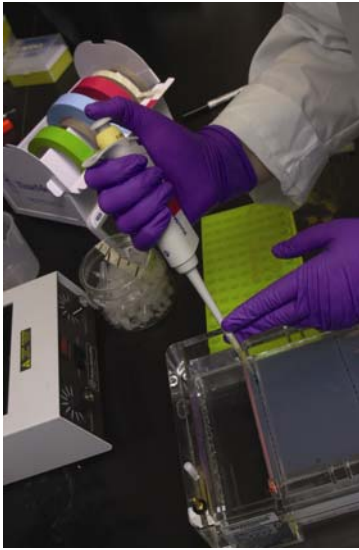
- "Spine Staple," invented by Eric Wall and Donita Bylski-Austrow
- "A Non-invasive Diagnosis of Early, Sub-Clinical Renal Injury," developed by Prasad Devarajan
- "Facile Assay of Fat Absorption," developed by James Heubi.

*See Validation Grants, page 4*

*Through the quarterly newsletter of the Cincinnati Children's Office of Intellectual Property and Venture Development, you will be able to keep abreast of some of the recent happenings in our office. We will update you regarding significant agreements between Cincinnati Children's and industry - including sponsored research, collaborative research, and licenses. We will advise you about patents that recently issued, and give you a preview of some of the newer technologies that we have available for licensing opportunities.*

*Of course, this newsletter is just a summary of items. You will want to regularly visit our web site to get more information.*





## *Cincinnati Children's Signs Licensing Agreement With itCube*

### *Agreement Demonstrates Benefits of Partnerships Between Ohio Businesses and Researchers*

Cincinnati Children's signed a licensing agreement with itCube, Inc., an Ohio-based company, to market customized, web-based software for researchers. The license agreement gives itCube exclusive worldwide distribution rights and marks the first time Cincinnati Children's has commercially licensed internally developed software. In addition, the agreement demonstrates the benefits of partnerships between Ohio businesses and researchers at Cincinnati Children's and the University of Cincinnati – an outcome envisioned last fall when Ohio's Third Frontier Project announced the investment of \$25.2 million in the Center for Computational Medicine.

The Center brings together, in one location, researchers from a number of disciplines at Cincinnati Children's and the University of Cincinnati College of Medicine, including human genetics, informatics, biostatistics, epidemiology, environmental health, mathematics, medicine and biomedical engineering. Investigators will collaborate with commercial partners to translate research discoveries into cutting-edge biotechnology and improved clinical treatments.

“When the Third Frontier decided to invest in the Center for Computational Medicine, it did so with the expectation that new technologies and Ohio jobs would be created,” says Keith Johnson, licensing coordinator in the Office of Intellectual Property and Venture Development at Cincinnati Children's. “While the technology licensed to itCube preceded the Third Frontier investment, the agreement illustrates that the Center for Computational Medicine can be

successful in commercializing bioinformatics technologies.”

The software applications covered by the agreement were developed under the leadership of **John P. Pestian, PhD**, director of the Center for Computational Medicine, in collaboration with several investigators at Cincinnati Children's and UC.

These applications are currently used by faculty and staff in the General Clinical Research Center, Tissue Procurement Facility and divisions of Pathology, General and Community Pediatrics, Critical Care Medicine and Pediatric Rehabilitation at Cincinnati Children's and UC. Select applications also are being used at Duke University and TriHealth, a partnership of Good Samaritan and Bethesda North hospitals in Cincinnati.

In addition to creating commercially viable software, investigators and business partners in the Center for Computational Medicine use sophisticated computers to analyze more than three billion complex pieces of information found in each of the trillions of cells in the human body. This analysis, using research and tools from the fields of genetics, systems biology and information and computer sciences, will result in fundamental discoveries about diseases that affect children and adults.

The Center also positions Ohio to capitalize on future technology opportunities by enhancing Ohio's computational infrastructure. It is expected to create more than 200 new jobs in the next five years and have a \$240 million direct impact on the region's economy.

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## *Licensee Updates: BTG Sublicenses Cincinnati Children's Hemofiltration Technology; Large Scale Biology Preclinical Trials to Begin*

**BTG**, the global technology commercialization company, announced that it has successfully licensed patents covering a hemofiltration device developed at Cincinnati Children's to international dialysis product manufacturer Gambro Renal Products. BTG acquired the rights to commercialize this intellectual property from Cincinnati Children's in November 2002.

The technology was originally developed by a team of researchers led by **John Bissler** at Cincinnati Children's. Through computerization, the device automates adjustments to an ongoing hemofiltration session, reducing the need for medical supervision and the possibility of human error.

In addition to improving existing methods for the treatment of acute renal failure (ARF), it is anticipated that this technology may further the development of the home dialysis market. The incidence of ARF is high, nearly 200 cases per million population in developed countries.

"We are pleased that Gambro, a globally recognized health care leader, has taken a license to this innovative technology from Cincinnati Children's, and we look forward to offering the rights to this technology to other players in the hemofiltration industry," said Ruth Sutherland of BTG's Strategic Business Development Group. "This builds on BTG's well-established history of delivering innovative medical technologies to industry."

"We believe this technology will bring significant improvement to hemodialysis and hemofiltration processes," said Joseph Fondacaro, Director of the Office of Intellectual Property & Venture Development for

Cincinnati Children's. "The license signifies the value of this technology and is recognition of the public benefit arising from Cincinnati Children's innovations."

"Gambro looks forward to exploring the opportunities for incorporating aspects of the licensed technology into its next generation of renal replacement equipment," said Annemarie Gardshol, senior vice president and head of marketing for Gambro Renal Products.

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**Large Scale Biology Corporation's** exclusive license agreement with Cincinnati Children's for the development of a drug, to treat a leading cause of death in the United States, has entered an initial development stage.

**Dr. Greg Grabowski**, co-authored a recent article published in the American Heart Association journal, *Arteriosclerosis, Thrombosis and Vascular Biology*, describing decreases in atherosclerotic plaque in mice on high fat diets when lysosomal acid lipase (LAL) was administered. Coronary and aortic plaque were eliminated in early stages and reduced quantitatively and qualitatively in advanced stages.

The initial research phase will be conducted at Cincinnati Children's Hospital Medical Center under the direction of Dr. Grabowski. Large Scale Biology Corporation will produce LAL for clinical applications as well as seek commercial partners for the drug.

Large Scale Biology uses functional genomics, biomanufacturing and proteomics technologies to develop and manufacture drugs, vaccines and diagnostics for effective treatment of disease.





Intellectual Property and Venture Development  
3333 Burnet Avenue  
Cincinnati, OH 45229-3039  
[www.cincinnatichildrens.org/ip](http://www.cincinnatichildrens.org/ip)

## At the Intersection of Research and Industry

To be added to the email List for TECH NEWS, please contact Keith Johnson at [keithw.johnson@cchmc.org](mailto:keithw.johnson@cchmc.org).

### *Validation Fund Grants continued from page 1*

**The Spine Staple technology** is a novel system for treating scoliosis patients. It is designed to surgically install, through minimally invasive procedures, “staples” into the spine to correct spinal deformities as the patient matures and grows. Importantly, it does not leave the patient with a permanent spine fusion. Prototyping has been done on an animal model, with positive results. The CTVF funding will allow for further prototyping and the initiation of the FDA approval process to begin using the device in a clinical study.

The second CTVF recipient is the **renal diagnostic technology**. Ischemic kidney injury leading to acute renal failure (ARF) is a frequent complication of cardiac surgery and kidney transplantation. Current

diagnostic techniques are lacking because kidney function is already hindered before a positive test result ensues. This new diagnostic method, which measures a protein called NGAL, can detect renal injury through a simple urine sample within hours of the injury. The CTVF funding will allow for additional human testing to go forward.

Along with the University of Cincinnati, **James Heubi** at Cincinnati Children’s is developing a **facile assay of fat absorption**. Fat malabsorption is commonly associated with cystic fibrosis, pancreatitis, and many other liver diseases. The current method of tracking malabsorption takes several days and is cumbersome. This new diagnostic technology will reduce that time to a few hours. CTVF funding will validate this new method in humans and move the technology toward the marketplace.

### *GSK to Build Facility for Rotarix® - Rotavirus Vaccine Invented at Cincinnati Children’s*

Cincinnati Children’s licensee AVANT Immunotherapeutics, Inc. announced that GlaxoSmithKline (GSK) plans to build a 450 million euro facility in Belgium. This facility will be used in part to develop, produce and conduct quality control on Rotarix®, the rotavirus vaccine on which AVANT and GSK have partnered. The facility is expected to come online in 2005.

Rotarix® is a two-dose, oral rotavirus vaccine originally invented in the Gamble Program at Cincinnati Children’s and licensed to AVANT. GSK obtained development and commercialization rights to Rotarix®, and is currently conducting Phase III trials of Rotarix® in 60,000 infants in Latin America and South East Asia. Based on the positive results from this trial, as well as data from

previously vaccinated children, GSK plans to market Rotarix® in Europe and other international markets before seeking approval to market in the United States.

Rotavirus infection causes potentially life-threatening diarrhea and dehydration in infants. It kills a child every minute in the developing world and causes over two million hospitalizations globally.

The vaccine now called Rotarix® was discovered in the 1990s by **Richard Ward, PhD** and **David Bernstein, MD**. It was licensed by Children’s to AVANT in 1995.

Dr. Ward has continued his work in the area of rotavirus vaccines. Along with **Anthony Choi, PhD**, he is developing a second generation rotavirus vaccine – known as VP6. Cincinnati Children’s is actively seeking corporate partners to assist with further development of the VP6 rotavirus vaccine.



Intellectual Property and Venture Development  
MLC 7032  
3333 Burnet Avenue  
Cincinnati, OH 45229-3039

Phone: 513-636-4285  
Fax: 513-636-8453

