

BAYLOR LICENSING GROUP ANNUAL REPORT

2013

Baylor
College of
Medicine[®]





Welcome to the Baylor Licensing Group (BLG) Annual Report for FY 2013. I hope you find the report to be informative and useful, and I hope that it illustrates the important role that commercialization plays in catalyzing the impact of the fantastic research being conducted at Baylor College of Medicine. BLG operates at the interface between the academic culture and the commercial culture, and I'm proud that our team is able to successfully bridge the two cultures to reach commercial agreements that open the door for the development of some of the college's most promising discoveries. The BLG team takes a pragmatic approach by seeking agreements that offer the opportunity for mutual gain. Our track record demonstrates an ability to get the job done and the deal closed.

MESSAGE FROM THE BLG DIRECTOR

We're at a very unique and exciting place in the college's history, and we're working on initiatives to increase the scope of the college's commercialization activities. BLG's activity level is growing rapidly—the pace of new disclosure submissions is steadily increasing, patent filings are up which builds the college's pool of proprietary intellectual property, and our deal flow remains strong. We've instituted a new program to produce an Invention Disclosure Analysis for newly submitted disclosures to provide better, more consistent service to BCM faculty. This past fiscal year, we negotiated and closed a landmark series of agreements with Celgene around the development of novel T-cell-based therapeutics against cancer that utilize chimeric antigen receptors (CARs) to direct the activity of these immune cells against their target molecules on the surface of tumor cells. You can read more about this transaction in the Agreement Highlights section of this report.

I'm personally very excited about the fact that Baylor College of Medicine is continuing to expand its efforts to support the internal development of promising new therapeutic and device technologies. The Alkek Pilot Projects in Experimental Therapeutics program, administered through the Dan L. Duncan Institute for Clinical and Translational Research (ICTR), provides an important source of funding to conduct key proof-of-concept experiments on some of the college's most promising approaches to the treatment of a variety of disease conditions. The launch of the Center for Drug Discovery will open new routes for the development of therapeutic molecules against novel targets for intervention. Programs like these will serve to strengthen the number and quality of the college's research discoveries that have a real chance to make the transition from the laboratory to the clinic. At a time when the pharmaceutical and biotech industries are cutting back on their internal research and development efforts, opportunities are being created for academic discoveries to fill the pipeline of the next generation of medicines. Baylor College of Medicine is well-positioned to leverage this opportunity.

BLG is charged with one of the most interesting and exciting tasks at the college—working with the college's researchers to try to find the right commercial partner to support development of their scientific advances. We are poised to enter a period of strong growth and greater impact from our commercialization activities.

- Michael Dilling

Disclosures on the Upswing!

Last fiscal year, technology disclosure submissions reached 116, reflecting a period of consistent growth over the past several fiscal years and an increase of 28 percent vs. FY 2011. During FY '12, BLG launched a new online disclosure submission application to streamline and simplify the process for submitting disclosures. We more recently launched a new Invention Disclosure Analysis (IDA) program, whereby faculty who submit disclosures are provided with an analysis of the technology that examines factors such as prior art, the commercial market, and the maturity of technology. The IDA concludes with a commercialization plan for the disclosed technology. BLG Director Michael Dilling commented, "The goal of the new IDA process is to provide better service and timely feedback to faculty who submit disclosures, and to do so in a consistent, easy-to-interpret format." The IDA process also helps BLG project managers make informed decisions about setting priorities for their efforts to land commercial licensees for inventions.

THE YEAR IN REVIEW

Planting Seeds for Future Success

New License Transactions

We signed 43 new licensing transactions this past fiscal year. Of these agreements, 19 were exclusive and 19 were non-exclusive, which is consistent with trends

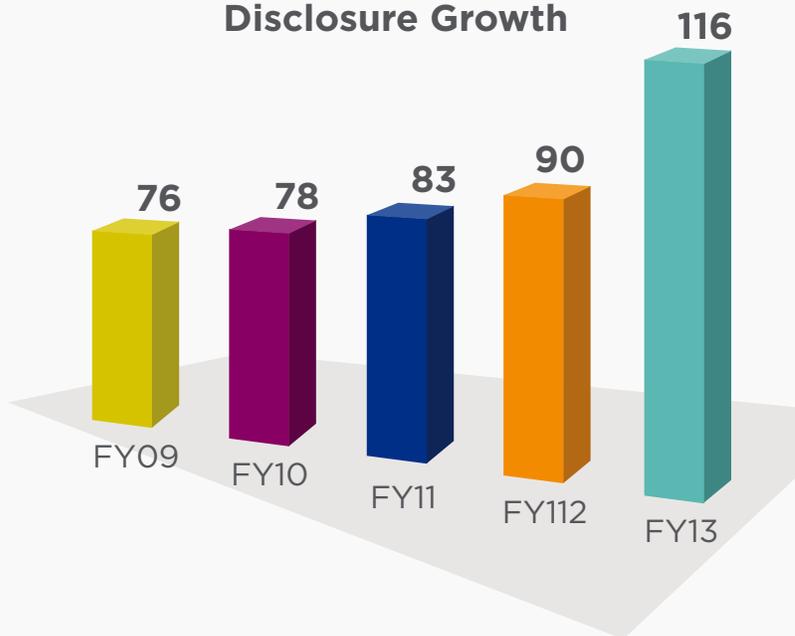
from past years' licensing activity. We also were involved in the negotiation of a number of non-licensing transactions that added value to the college. The college has always been a strong generator of non-patented research tool technologies (knockout mice, antibodies, specialized cell lines and vectors, etc.) that add value to the research and development programs of pharmaceutical and biotech companies, and a number of these tools are quite popular in the commercial sector. As the college ramps up its efforts to support the internal development of new experimental therapeutics, vaccines, and devices, we would anticipate an increase over time in exclusive license transactions.

Licensing Revenue Distribution

Baylor College of Medicine's Policy on Patents & Other Intellectual Property provides for the distribution of net license income (gross license revenue less 15 percent to support BLG operations, and reimbursement of any unreimbursed legal expenses) to inventors (40 percent of net), their departments (30 percent of net) and the Baylor General Fund (30 percent of net). During FY '13, we distributed \$3.8 million to inventors, \$2.7 million to departments, and \$2.9 million to the Baylor General Fund. We also distributed \$1.5 million to third party funding sources that supported the development of technologies that we licensed, and we distributed \$765,000 to other institutions with whom we co-own technologies. As is always the case, the vast majority of license revenue is associated with a small number of licenses, several of which were signed long ago. This trend (most revenue being associated with a few key deals) is true not just at BCM but across academia. It can take a decade or more from the date that a license agreement is signed until the college begins to receive substantial revenue from the sale of licensed products based on the licensed technology.

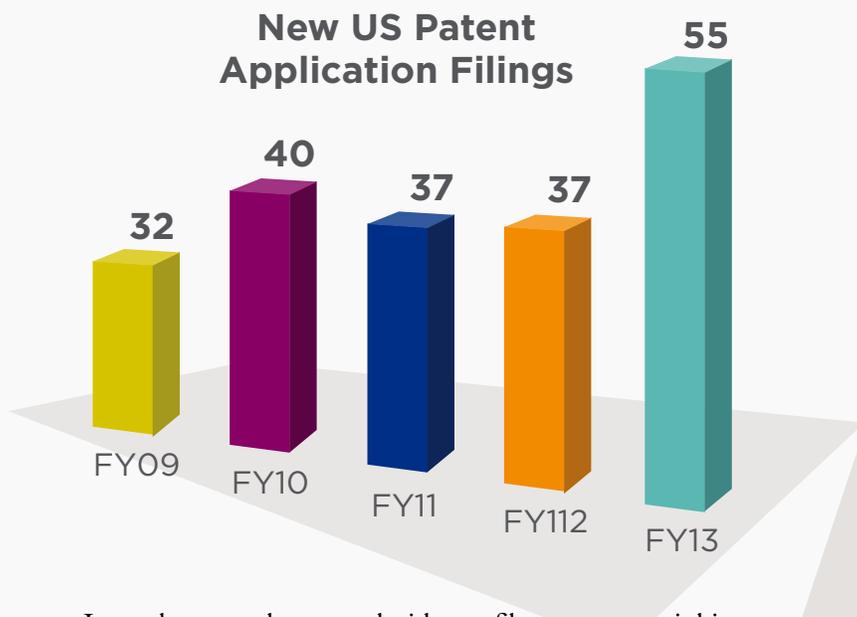
Our top technologies in terms of licensing revenue for this past fiscal year were: (1) antimicrobial coatings for urological devices, catheters, and other implantable medical devices, and (2) a platform technology for the development of tumor-fighting cytotoxic T-cells containing chimeric antigen receptors (CARs).

Disclosure Growth



Patent Filings

We filed 55 new patent applications last fiscal year (the highest in six years), and this increase correlates with the increase in disclosure count and with the fact that many of the new disclosures were devoted to potentially novel therapeutic methods and compositions. The college takes a financial risk when we decide to file a patent on a new invention that may never see the clinic, however, in order to find commercial partner who will assume the risks associated with the commercial development of a risky, preclinical, early-stage technology, we frequently move forward with the patent filing and prosecution process.



In each case when we decide to file, we are weighing the likelihood that the technology represents a good licensing candidate, and that having a patent application will be necessary to interest a licensee. We believe that Baylor College of Medicine's pool of proprietary, patented, intellectual property should grow as the college continues to augment its efforts to support the development of new therapies and devices. We must continue to be judicious and diligent in our efforts because patent filing and prosecution is an expensive proposition—it can cost \$30,000 or more to file and prosecute a patent to issuance in the U.S.; and prosecuting even a conservative foreign portfolio can be \$100K or more.



NEW FACES ON THE BLG SCENE

Brian Phillips, Ph.D.

Licensing Associate II

Brian Phillips works as a manager of novel technologies developed at Baylor College of Medicine and is responsible for the Departments of Pediatrics, Pathology & Immunology, Pharmacology, Physical Medicine and Rehabilitation, Radiology, Dermatology, and Family and Community Medicine.

Brian joined the Baylor Licensing Group in May 2012 after working as a Licensing Associate for The Texas A&M University System's Office of Technology Commercialization. At Texas A&M he led commercialization activities in the areas of biomedical sciences, agricultural sciences, and chemical engineering. Prior to this, Brian worked as a Licensing Associate at Rice University's Office of Technology Transfer. In his role as a Licensing Associate, Brian has executed over thirty license agreements worth more than \$1.5 million in upfront fees and spin-out seven startup companies. Brian is an active member in the Association of University Technology Managers, the Licensing Executives Society, and serves on the review committee for the Texas Emerging Technology Fund - Gulf Coast Regional Center of Innovation and Commercialization.

He received a Ph.D. in Biomedical Sciences with the Genes and Development Graduate Program at the University of Texas MD Anderson Cancer Center and a B.S. in Biology from the University of Houston where he graduated cum laude and as a member of the Honor's College.

AGREEMENT HIGHLIGHTS

On March 19, 2013, BCM signed an exclusive multiyear research and collaboration agreement and a platform technology license agreement with Celgene Corporation that launches the commercial development of novel immunotherapies involving manipulated T-cells that express chimeric antigen receptors (CAR CTLs). The licensed CAR CTL platform represents the collective research efforts of a team of principal investigators at the Center for Cell and Gene Therapy (CAGT), led by Director Malcolm Brenner. The CAR CTL approach involves the genetic modification of a patient's T-cells, a strategy that utilizes the expression of the chimeric antigen receptor to direct the manipulated T-cell to target and destroy tumor cells. The CAGT team is developing a number of different CAR CTL approaches that can potentially be used as weapons against a variety of tumor types.

Concurrent with the execution of the Celgene-BCM agreements, Celgene and its partner bluebird bio entered into a global strategic collaboration, such that this unique multiyear research and development alliance will leverage the strengths and talents of all three parties: (1) the expertise of the CAGT team to design, develop, and test novel CAR CTLs and guide them toward clinical entry, (2) bluebird bio's expertise in personalized gene therapy drug development with a focus on leveraging its proprietary lentiviral vector platform, and (3) Celgene's expertise and resources around conducting large-scale clinical trials and the development of commercially viable, GMP compliant manufacturing processes. Under the terms of the agreements, Celgene has an option to license any CAR CTL product emerging from the collaboration after the completion of a phase I clinical trial. Bluebird bio

CELGENE, BLUEBIO BIO, AND THE CENTER FOR CELL AND GENE THERAPY COMBINE STRENGTHS AND RESOURCES TO DEVELOP T-CELL BASED THERAPIES FOR CANCER

will guide the commercial research and development of these products through the phase I trial process. The collaborations aim to yield new products that could represent future "standards of care" for treatment of both solid tumors and hematological malignancies. "The

genetic manipulation of autologous T-cells is a new frontier in oncology, one that shows early promise in emerging clinical trials," said Tom Daniel, President, Research and Early Development, at Celgene. "We see strong prospects for this collaboration between Celgene, bluebird bio and Baylor College of Medicine's experienced leaders in this emerging field, led by Dr. Brenner, to advance this innovative approach to intractable problems in oncology."

CAGT Director Malcolm Brenner stated, "The data on T-cell therapy for the treatment of multiple different cancers is extremely encouraging; this exciting collaboration provides a unique path to effectively develop chimeric antigen receptor-modified T cells to change and advance available treatments for cancer patients worldwide."

"The goal of technology transfer is to open doors for the commercial development of our most promising therapeutic approaches," stated Michael Dilling, BLG Director. What makes the Celgene, bluebird bio, CAGT collaboration so exciting is the fact that this combined, talent-rich team is aligned and working toward the common goal of bringing highly effective novel personalized cancer therapies to patients worldwide. All of the components necessary for the successful development and commercialization of these promising CAR CTL approaches are assembled and working together. We look forward to the day that these therapies are widely available to help patients."

STEMMED, LTD., LICENSES STAT3 INHIBITORS

Local company developing novel compounds against a key cancer target

In July 2012, StemMed, a local company founded by BCM faculty members Drs. David Tweardy (Department of Medicine), Michael Lewis (Lester and Sue Smith Breast Center) and former BCM faculty member Jenny Chang (now with Houston Methodist Research Institute), executed an exclusive license agreement with the college to open the door for commercial development of a series of STAT3 inhibitor compounds. These compounds, developed in the Tweardy laboratory at BCM, are directed against signal transducer and activator of transcription (STAT) 3, a regulatory protein that is amplified in a wide variety of cancers, including breast, prostate, lung, head and neck, colon, liver and pancreas. STAT3 plays a pivotal role in promoting growth and inhibiting apoptosis (programmed cell death) in these tumor cells, and therefore, the development of a compound that specifically and selectively inhibits STAT3 would be a welcome addition to the arsenal against cancer. Developing drugs that are specific to a protein like STAT3, which is a member of a multi-gene family, is not a trivial process, and the Tweardy lab employed a computational structural biology-based approach to attack the problem. Their analysis revealed the existence of a binding site that facilitated the identification of compounds that specifically bind to and inhibit STAT3. StemMed is now coordinating the commercial development of the most promising class of compounds identified by the Tweardy team, and will shepherd the compounds in to the clinic. “The development of a specific and selective STAT3 inhibitor has been a long-term professional goal of mine, and the license of these compounds in to StemMed moves us one important step closer to getting them in to patients,” said Dr. Tweardy.

ATCC MASTER AGREEMENT

BCM poised to benefit from commercial use of deposited materials

In January of 2013, BCM finalized a master license agreement with the American Type Culture Collection (ATCC) that allows BCM to receive royalties on sales of biological materials deposited at ATCC. Previously, ATCC had maintained materials deposited by BCM faculty and made them available for sale to the non-profit and for-profit research communities. However, until now, BCM did not profit from such sales. Following the execution of the Master Deposit Agreement, BCM will now receive royalties on the sales of such deposited materials. In return, BCM will share a portion of licensing revenue received from for-profit entities that have purchased the materials from ATCC following the execution of a license agreement from BCM. The agreement provides depositors financial incentives to deposit materials at ATCC and streamlines the process for future deposits.

TyRx, INC., EXPANDS MARKET AND INTRODUCES BIORESORBABLE AIGIS_{RX} R ANTIBACTERIAL ENVELOPE

The scope of a successful licensing relationship grows

TyRx, Inc., a New Jersey-based company, commercializes innovative, implantable combination drug-plus-device products focused on infection control, including the AIGIS_{RX} Antibacterial Envelope, designed to reduce surgical site infections associated with Cardiac Implantable Electronic Devices (CIEDs). The AIGIS_{RX} Antibacterial Envelope, a combination drug-device product, utilizes novel anti-bacterial coating technologies and patents jointly developed by Dr. Rabih Darouiche at Baylor College of Medicine and Dr. Issam Raad at The University of Texas MD Anderson Cancer Center. The novel coating technologies contain the antimicrobial agents, rifampin and minocycline, which have been shown to significantly reduce surgical site infection by pathogens responsible for the majority of CIED infections, including bacteria such as MRSA.

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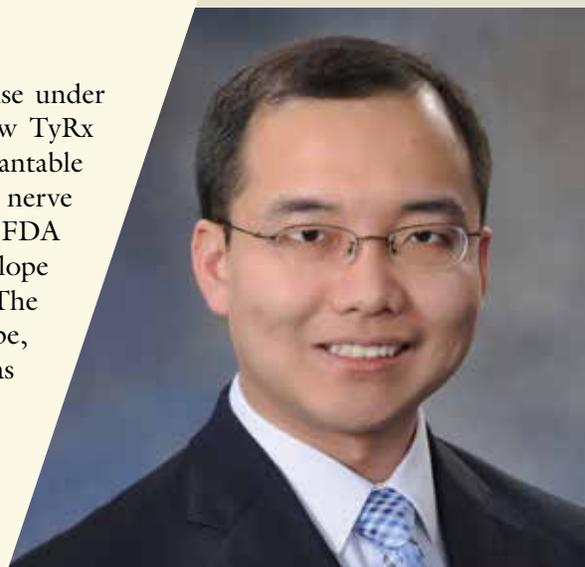
AGREEMENT HIGHLIGHTS CONTINUED

In June 2013, BCM and TyRx Inc. agreed to an expanded field of use under their current license agreement. This field of use expansion will allow TyRx to sell its signature antibacterial products in new markets such as implantable infusion devices (i.e., drug pumps) and pulse generators (i.e. vagus nerve stimulators). Additionally, TyRx recently announced that it had received FDA clearance to market its fully resorbable AIGISRx R Antibacterial Envelope for use with pacemakers and implantable cardioverter defibrillators. The AIGISRx R is TyRx's second-generation antibacterial mesh envelope, which provides device stabilization and infection reduction, but also has the added benefit of being a fully resorbable product.

THE TRAMP MOUSE MODEL AND CELL LINES

A research tool's popularity marches on

The Transgenic Adenocarcinoma Mouse Prostate (TRAMP) model is a transgenic line of C57BL/6 mice that develops histologic prostatic intraepithelial neoplasia by 8 to 12 weeks of age which progresses to adenocarcinoma with metastasis by 24 to 30 weeks of age. TRAMP was established using the minimal -426/+28 rat probasin promoter to target expression of the simian virus 40 large T antigen to prostatic epithelium. Three cell lines were established from the primary tumor in the prostate of a PB-Tag C57BL/6 (TRAMP) mouse and designated TRAMP-C1, TRAMP-C2, and TRAMP-C3. The mouse model and cell lines are useful for companies involved in prostate cancer research and are deposited at, and available for sale from, the Jackson Laboratories and ATCC, respectively, following a license for use from BCM. These materials were developed in former BCM faculty member Norman Greenberg's laboratory in collaboration with the University of Manitoba in the 1990s. BCM has licensed the mouse model more than 30 times over the years and the cell lines more than 40 times and is still collecting royalties on 18 of those agreements. There is continued interest in industry for both the mouse and cell lines and we signed a number of new TRAMP agreements in FY '13. The TRAMP mice and cell lines provide a great example of a non-exclusive licensing strategy that has led to widespread commercial use and adoption of a popular model.



NEW FACES ON THE BLG SCENE

Qinxu "Andy" Guo, Ph.D. Licensing Assistant

Andy joined the Baylor Licensing Group in 2013 and is responsible for evaluating and marketing inventions. He received a Ph.D. in Translational Biology and Molecular Medicine from Baylor College of Medicine (BCM). While at BCM, Andy conducted neuroscience research at the interface of basic science and clinical medicine, led a clinical research project, and worked closely with BLG to promote technology commercialization. Andy is a member of the Association of University Technology Managers. Andy stated, "I am very excited to be a member of the BLG team, because it gives me the unique opportunity to bridge the bench, the bedside, and the market, and to maximize the impact of BCM inventions."



PROFILES IN INNOVATION

A look at some of the most promising approaches under development at BCM to diagnose and treat disease

CHRISTINE BEETON, PH.D.

Department of Molecular Physiology

Potassium channel modulation as a therapeutic strategy for chronic inflammatory disease

Potassium channels play crucial roles in regulating the function of a large number of electrically excitable cells and non-excitable cells. Potassium channels are highly diverse in their subunit composition and physiological function, but each type has restricted tissue distribution. Therefore, it is possible to selectively target one channel type to modulate cell activity in a tissue-specific manner, which makes potassium channels attractive targets for drug development. Dr. Christine Beeton, Associate Professor of Molecular Physiology and Biophysics, and her team at Baylor College of Medicine aim to identify and target potassium channels for chronic inflammatory diseases such as rheumatoid arthritis (RA), multiple sclerosis, asthma, and myotonic dystrophy type I.

RA is a chronic inflammatory disease that affects 1.3 million people in the United States and there is no cure at this time. Current therapeutic approaches target the immune response. However, these drugs can induce severe side effects associated with immunosuppression that limit their long-term use and are inappropriate for patients with chronic infections. Therefore, novel therapies that target non-immune pathways are highly desired and would be of great benefit to patients. Dr. Beeton's group has conducted successful proof-of-concept studies on novel RA treatment.

Dr. Beeton's team identified KCa1.1 as the major potassium channel in RA-activated fibroblast-like synoviocytes (RA-FLS). FLS are a cell type located inside joints in the synovial membrane of most joints, and play a key role in perpetuating inflammation and contributing to cartilage destruction in RA. Treatment of RA-FLS with paxilline (PAX), a specific KCa1.1 small molecule inhibitor, significantly inhibits the proliferation and invasion of RA-FLS. Furthermore, targeting of this channel halts the progression of RA in an animal model. Utilizing a combinatorial

approach of chemical synthesis, electrophysiology, and pharmacokinetics, the Beeton group, in collaboration with the Horrigan and Pedersen groups at BCM, is currently developing a targeted drug delivery system for PAX that can be used therapeutically to inhibit RA-FLS in inflamed joints.

In addition, Dr. Beeton previously made significant breakthroughs to target voltage-gated potassium channels in T-cell mediated autoimmune diseases, including multiple sclerosis and its animal model counterpart, experimental autoimmune encephalomyelitis (EAE).

Applications/Advantages

- New therapies that target potassium channels in chronic inflammation disease can be an alternative strategy to immunosuppression or co-administered with current treatments
- Potassium channel modulators are already clinically used as drugs for the treatment of type-2 diabetes and cardiac arrhythmia, supporting its safety and efficacy
- The primary investigator has more than a decade of research experience on potassium channels and their impact on autoimmune chronic inflammatory diseases
- Expertise in potassium channel blockers and autoimmune diseases provides a scientific framework for extending drug design beyond RA
- Comprehensive, multidisciplinary drug design, evaluation, optimization strategy in collaboration with other leading experts in the field
- Previous research findings have been published in numerous high-profile scientific journals and cited by numerous colleagues in the field

BLG Project Manager

Terese Rakow, Ph.D. (trakow@bcm.edu)



NEW FACES ON THE BLG SCENE

Veronica Hinojosa Intellectual Property Coordinator

Veronica joined the BLG team in 2013. Veronica assists BLG team members with administrative matters associated with intellectual property prosecution and maintenance. She serves to coordinate communication with outside legal counsel, inputs legal correspondence into the BLG database, and she manages compliance issues associated with federally-funded grants. Veronica has held past positions as a legal assistant.



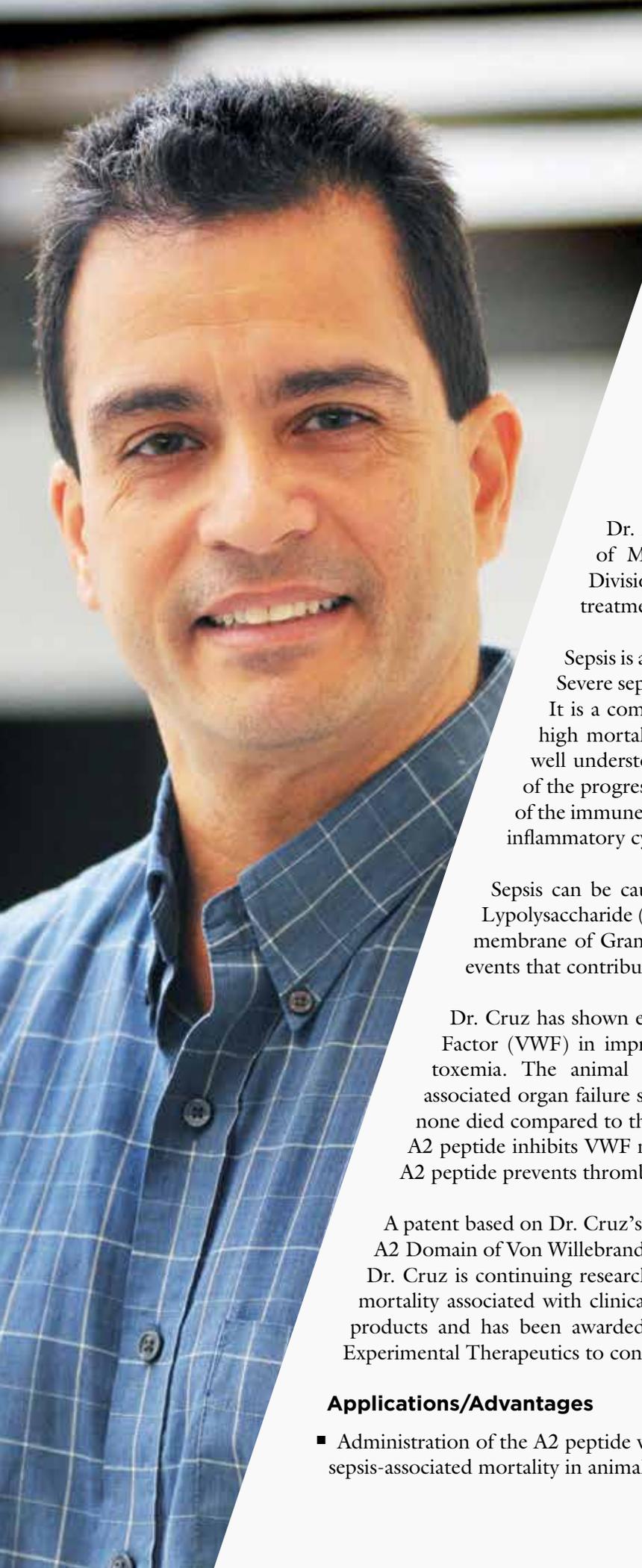
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MIGUEL CRUZ, PH.D.

Department of Medicine
Division of Thombosis

Developing a novel treatment for systemic inflammation

Dr. Miguel Cruz, an Associate Professor in the Department of Medicine at Baylor College of Medicine, Thrombosis Division, has devoted his research efforts to developing a treatment for systemic inflammation (*i.e., endotoxemia, sepsis*).

Sepsis is a systemic inflammatory response to an infection or insult. Severe sepsis is a serious public health concern in the United States. It is a common diagnosis among critically ill patients and carries a high mortality rate. However, the pathophysiology of sepsis is not well understood, and afflicted patients deteriorate rapidly as a result of the progressive failure of multiple organs. In sepsis, hyperactivation of the immune response leads to the excessive production of various pro-inflammatory cytokines and cellular injury.

Sepsis can be caused by a non-infectious or infectious insult, including Lypolysaccharide (LPS or endotoxin). LPS is a component of the outer cell membrane of Gram-negative bacteria that can initiate a parallel cascade of events that contribute to the clinical manifestations of sepsis.

Dr. Cruz has shown efficacy of the isolated A2 domain of the von Willebrand Factor (VWF) in improving survival of mice with lethal LPS-induced endotoxemia. The animal model closely models microvascular thrombosis and associated organ failure seen with sepsis. The pretreated animals became sick but none died compared to the controls. In vitro, Dr. Cruz has observed that purified A2 peptide inhibits VWF mediated platelet adhesion to fibrin, which suggests that A2 peptide prevents thrombus formation.

A patent based on Dr. Cruz's discovery, titled "Treatment of Medical Condition With A2 Domain of Von Willebrand Factor," has been issued to Baylor College of Medicine. Dr. Cruz is continuing research on A2 peptide in efforts to reduce the morbidity and mortality associated with clinical conditions generally caused by endotoxin or bacterial products and has been awarded funding from the Alkek Award for Pilot Projects in Experimental Therapeutics to continue the effort towards clinical application.

Applications/Advantages

- Administration of the A2 peptide was associated with a dramatic, complete reduction in sepsis-associated mortality in animal models



- This novel therapy has applications in sepsis and inflammation resulting from either an infectious or non-infectious insult
- The A2 peptide therapeutic molecule presents low immunogenicity and a low hurdle to tolerance
- Additional ongoing work will examine the mechanism and consequences of administration of the A2 peptide on a panel of biomarkers associated with systemic inflammation in humans

BLG Project Manager

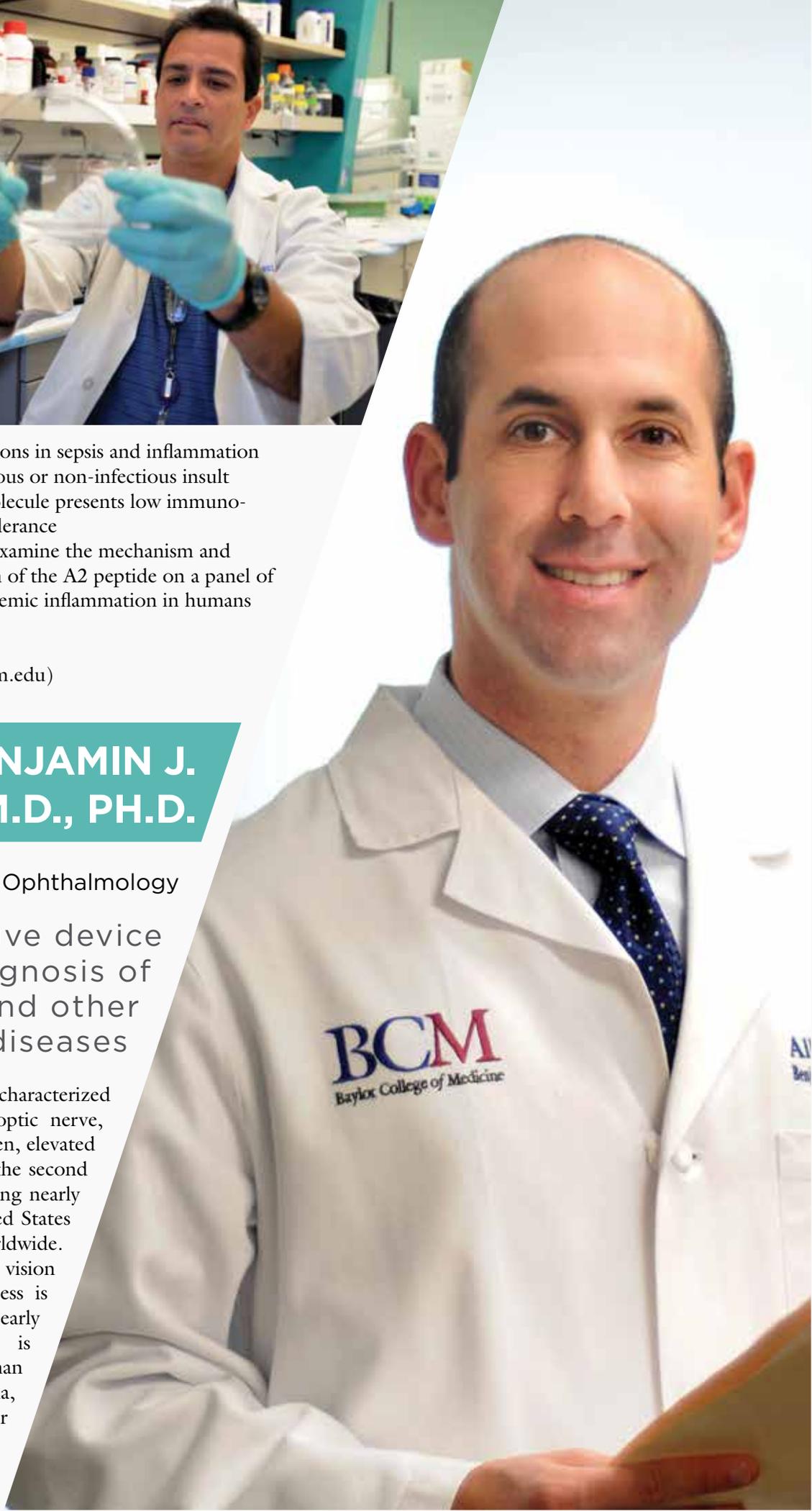
Mercy Chen, Ph.D. (mschen@bcm.edu)

BENJAMIN J. FRANKFORT, M.D., PH.D.

Department of Ophthalmology

A non-Invasive device for early diagnosis of glaucoma and other ophthalmic diseases

Glaucoma is an ocular disorder characterized by progressive damage to the optic nerve, retinal ganglion cell loss, and, often, elevated intraocular pressure (IOP). It is the second leading cause of blindness, affecting nearly 3 million individuals in the United States and more than 60 million worldwide. Glaucoma is often treatable and vision loss likely preventable, but success is significantly contingent upon early diagnosis. Contrast sensitivity is one of the first aspects of human vision to be affected by glaucoma, which can potentially be used for





early diagnosis. However, current testing methods are problematic due to their subjective nature, long testing time, the need for patient cooperation and concentration, and limitations related to lighting conditions. Thus, there is a significant need for improved methods of glaucoma detection, and especially objective functional assessment in the earliest stages of disease.

Dr. Benjamin J. Frankfort, Assistant Professor of Ophthalmology, and his team at Baylor College of Medicine are making significant breakthroughs in improving early detection of this prevalent and progressive disorder. The Frankfort group has recently developed a new device, the Human Optokinetic Contrast Detector (HOCD), which overcomes many of the limitations present in current devices/tests. The HOCD is an automated system, with a customizable software program, which primarily quantifies contrast sensitivity. It is rapid and objective and can be used under multiple lighting conditions. The HOCD has been validated in a glaucoma mouse model, and the prototype successfully measured contrast sensitivity of humans in multiple ages and visual acuities. As it measures contrast sensitivity, a key aspect of human vision, the HOCD is also a versatile device. It can potentially be used to: (1) diagnose glaucoma prior to other techniques, including clinical examination; (2) stage glaucoma; (3) diagnose/stage other ophthalmic diseases, including functional changes associated with diabetic retinopathy, cataract, neurologic disease with ocular manifestations, vision in preverbal or uncooperative children; and (4) assess contrast sensitivity or other visual function parameters in clinical trials or animal studies.

Dr. Frankfort was awarded funds from the John B. Carter, Jr. Technology Catalyst Fund administered by BLG to

construct a HOCD prototype to be used in a pilot clinical trial. The prototype built primarily with off-the-shelf materials is being finalized for transfer to the clinic for testing.

Dr. Frankfort's team has extensive experience and excellent expertise in glaucoma, clinical research, HOCD prototype design, software development and data analysis. They are dedicated to seeking out innovative solutions for the diagnosis of ophthalmic diseases and improving the ability of ophthalmologists to prevent, treat, and monitor those diseases.

Applications/Advantages

- Successfully validated prototype in both animals and humans
- Automated, objective test: no patient input required
- Usable in multiple lighting conditions
- Fast: Less than 2 minutes per eye
- Versatile: Potential to provide early diagnosis of glaucoma and other conditions
- Large market of interest: More than 18,000 ophthalmologists and 36,000 optometrists in the U.S.
- Research applications in animals including non-human primates

Technology Status

The HOCD device and testing protocol are being optimized for clinical trial.

BLG Project Manager

Terese Rakow, Ph.D. (rakow@bcm.edu)



MAURO COSTA-MATTIOLI, PH.D.

Department of Neuroscience

Developing compounds to enhance cognitive function

Memory is essential for human experience, and most of what we know about the world is stored as memory. There are two memory systems: short-term memory (STM), which lasts minutes, and long-term memory (LTM), which lasts for years or even a lifetime. Memories are stored in the brain as changes in the strength of the connections between neurons, and cognitive functions arise from the finely-tuned coordinated interactions of a large number of neurons widely distributed throughout the brain.

Since memory is at the center of our identity, the loss of memory leads to the loss of self. Learning and memory disorders strike the brain not only during development—for example, in Autism and Down syndrome—but also in the adult—for instance in Alzheimer’s disease, Huntington’s disease, Parkinson’s disease and aging. By 2030, the incidence of Alzheimer’s disease is expected to be 15 million in the U.S. Currently, there are no treatments that can prevent, delay or reverse the memory problems associated with Alzheimer’s or other cognitive disorders including Autism, Parkinson’s disease, Down Syndrome, PTSD, and addiction.

The lab of Dr. Mauro Costa-Mattioli, Assistant Professor of Neuroscience, has provided novel genetic, physiological, pharmacological, behavioral, and molecular evidence that the double-stranded RNA-activated protein kinase (PKR) negatively regulates memory storage. They have shown that genetic inhibition or pharmacological inhibition of PKR enhances learning and long-term memory



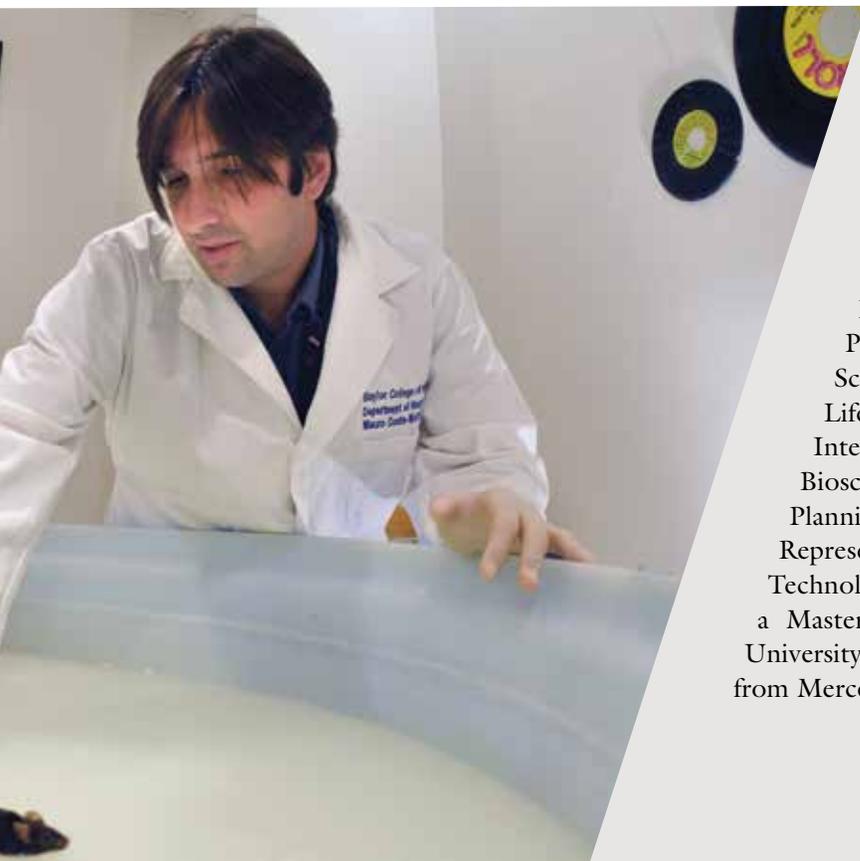
formation in mice. Likewise, they have developed a class of novel small molecule inhibitors, which block PKR (PKRi), and have shown that they enhance long-term memory storage. This evidence suggests that cognition may be enhanced in an individual by providing a therapeutically effective amount of the PKRi. Since the activity of PKR is altered in several neurological disorders, including Alzheimer's Disease, Parkinson's, and Huntington's disease, this kinase and inhibitor present a promising new treatment for cognitive dysfunction. His lab is the only lab in the world to provide two mouse models of enhanced cognition as well as a memory-enhancing drug.

Applications/Advantages

- Development of a novel, small molecule inhibitor of PKR, and demonstration that it enhances long-term memory formation
- Their studies address an important unmet medical need—Cognitive disorders (from Aging to Alzheimer's disease to PTSD)
- The Costa-Mattioli lab has already provided two mouse models of enhanced cognition and also molecules (drugs) that can enhance memory formation
- The inventors use a multidisciplinary approach that combines mouse and fly genetics, molecular biology, chemical biology, behavior, electrophysiology (at the single cell, synaptic and network level) imaging and drug discovery which will provide useful leads to treat cognitive dysfunction

BLG Project Manager

Lisa Beveridge (lisa.beveridge@bcm.edu)



NEW FACES ON THE BLG SCENE

Andrew Wooten, M.S.T, M.T.M

Executive Director,
Research Business
Development & Strategy

Andrew joined Baylor College of Medicine in 2012. He is affiliated with Baylor Licensing Group and the Dan L. Duncan Institute for Clinical and Translational Research. Andrew is creating strategies to facilitate the translation of BCM's biomedical innovation assets into real-world products, ventures and jobs. This includes building support programs and infrastructure. It also includes attracting stakeholders and commercial partners to support these efforts.

Prior to joining BCM Andrew held a variety of positions at the interface of academia and industry. Former positions include Director of Outreach and Commercialization with Gateway University Research Park; Vice President, U.S. West with Neri Capital Partners; Vice President and Managing Director of Health Science Ventures with Arizona Technology Enterprises; Life Science Project Manager, Office of Technology and Intellectual Property, University of Chicago; President, Ovo Biosciences; Director of Corporate Development and Strategic Planning, Avigenics/Synageva Biopharma; and Genetic Analysis Representative, Business Development, Applied Biosystems/Life Technologies. Andrew has a B.A in Chemistry from Berea College, a Master's of Science Technology in Biotechnology from the University of Georgia, and a Master's in Technology Management from Mercer University.

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