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The Georgia Bio Industry E-Newsletter

Newsletter Issue: March 2017

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Georgia Bio Newsletter Credits

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Letter from the President

Following a great night at our annual awards dinner, Georgia Bio is planning many activities for you and your colleagues in the upcoming months. Be sure to check our calendar and consider sponsorship opportunities, speaking roles and attendance at these programs.

We are busy with state and federal policy efforts, including improved tax policies and support of our STEM education programs under the Georgia BioEd Institute. The Institute is hard at work to improve the industry's workforce in Georgia, and continues to work towards expanding biotechnology training programs and equipment support for high school teachers. I invite you to join us at two upcoming programs to lend your support for the Institute:

Georgia BioEd has organized a biotech pavilion at the Atlanta Science Festival Exploration Expo on March 25th, and we invite our members to join us and show kids in our state the exciting work that our industry does. Click here for more information.

On April 10th, after watching a few exciting days of the Masters, you will be anxious to show off your own skills on the golf course! We invite you to join us at the 3rd Annual Swings Fore STEM Golf Outing at the Country Club of Roswell. Your sponsorship or attendance at this event will contribute directly to the Institute's programs and help us impact more kids across Georgia.

Also, be sure to attend the annual Academic & Industry Intersection Conference. It is always an excellent event and a chance for our industry leaders to coordinate with our research universities to continue growing life sciences in Georgia.

If you have not yet discussed the benefits of your membership with our VP of Member Relations, Stephen MacDonald, I encourage you to do so. Stephen will help you to customize and maximize the value of your membership.

I look forward to seeing you soon,

Russell

FDA Draft Guidance on Quality Metrics

Promoting Quality Culture in Pharmaceutical Industry

Datalynx

FDA defines its vision for the pharmaceutical industry in the 21st Century as "a maximally efficient, agile, flexible pharmaceutical manufacturing sector that reliably produces high quality drugs products with extensive regulatory oversight." The FDA has been working on solutions to encourage and support the modernization of pharmaceutical manufacturing. The quality metrics initiative is one of several approaches that hopes to support this vision.

Quality metrics are defined as an "objective measure of the quality of a process." Quality is a measure of a site's ability to manufacture product(s) fit for intended use. It can also measure the effectiveness of systems associated with the manufacture of pharmaceutical products, such as the pharmaceutical quality system. Current sources of quality metrics are sometimes fragmented, disparate, and/or incompatible. This is an important concern because quality metrics can be used to determine a drug's safety and effectiveness, monitor quality control systems, and drive continuous improvement efforts in drug manufacturing. These metrics are also used by the FDA to develop compliance and inspection policies and practices, as the FDA recognizes the importance of implementing a quality-driven culture when manufacturing and selling pharmaceutical drugs.

On July 28, 2015, the FDA released the anticipated draft guidance document, Request for Quality Metrics, which outlines the FDA's authority to require owners, operators, and manufacturers to provide records and information (upon request) that they many inspect under section 704 of the Federal Food, Drug, and Cosmetic Act. The type of manufacturers that need to submit records for inspection are specified in the draft guidance document. The draft guidance also details the types of data the FDA plans to request from manufacturers, how they plan on using the data, and which quality metrics it plans to calculate. The FDA will use quality metrics to assist with risk based inspection schedules as required under FDASIA, Title VII, section 707. Quality Metrics can also be used to segment and classify products, processes, and product manufacturers based on risk. The draft guidance document also recommends that manufacturers conduct robust quality measurements on their own products.

Using quality metrics for risk-based inspection scheduling can help to predict and mitigate drug shortages, improve the inspection process, and improve their evaluation of drug manufacturing and control operations. FDA hopes that establishing quality metrics will lead to a decreased surveillance inspection frequency for certain establishments. For instance, establishments that have highly controlled manufacturing processes have the potential to be inspected less often. Additionally, the robust quality metrics data can be used to "reduce the inspection frequency at an establishment." According to the guidance document, the FDA will request the following 10 baseline quality metrics from companies as part of its analysis:

- The number of lots attempted of the product.
- The number of specification-related rejected lots of the product, rejected during or after manufacturing
- The number of attempted lots pending disposition for more than 30 days.
- The number of out-of-specification (OOS) results for the product, including stability testing.
- The number of lot release and stability tests conducted for the product
- The number of OOS results for lot release and stability tests for the product which are invalidated due to lab error.
- The number of product quality complaints received for the product.
- The number of lots attempted which are released for distribution or for the next stage of manufacturing the product.
- If the associated annual product reviews (APRs) or product quality reviews (PQRs) were completed within 30 days of annual due date for the product.
- The number of APRs or PQRs required for the product

Effects of Non-Reporting

Section 704(a)(4)(A) of the FD&C Act authorizes FDA to request data and records from a person that owns or operates an establishment that is "engaged in the manufacture, preparation, propagation, compounding, or processing of a drug" in advance of an inspection. Providing data to the FDA will assist staff in preparing for drug quality surveillance inspections, thus improving their efficiency and effectiveness. If an owner, operator, or agent of a facility fails to produce records and information requested (based on section (a)(4) of the FD&C Act) within a reasonable timeframe, drugs from the facility may be deemed adulterated under section 501 of the Act and subjected to enforcement action. If a company or manufacturer fails to report quality data, the FDA "may elevate an establishment's predicted risk in FDA prioritization of inspects and may lead to an earlier inspection."

How Datalynx Can Help You Implement Quality Metrics

With 20 years of experience in regulatory compliance, Datalynx is poised to help you through the process of implementing quality metrics in the pharmaceutical manufacturing. We can help at any stage of the process by ensuring that the proper support is present to bring your team up to speed and meet the requirements of the FDA's standards for quality metrics.

Contributors: Manoj Karamchandani

Georgia Universities Team Up to Speed 'Translation' of Diabetes Research

Georgia Research Alliance

Discoveries about diabetes need to be put to work faster, according to the National Institutes of Health. So to help achieve that aim, Emory, Georgia Tech and Morehouse School of Medicine are teaming up in a new enterprise designed to accelerate the translation of diabetes research into new treatments and prevention strategies. Recent years have brought higher numbers of people diagnosed with diabetes than expected, and onset of the disease is happening earlier in life. A $2.5 million NIH grant to Emory created the multi-university enterprise, called the Georgia Diabetes Translation Research Center. The consortium will also bring in collaborators from Georgia State and the Atlanta Veterans Administration Hospital.
• Read the news release http://diabetes.emory.edu/research/GDTRC.html

The 2017 Golden Helix Awards Acknowledges the Best in Life Sciences

By Kristen Pappaterra, Georgia Bio

On January 26th, Georgia Bio hosted the Life Science Luminaries Gala Celebration and 2017 Golden Helix Awards at the Westin Atlanta Perimeter North. Each year, Georgia Bio recognizes achievement and honors excellence in the life sciences industry in Georgia. The evening played host to nearly 250 members of Georgia's life science community. Now in its 19th year, the annual awards event brings life science industry leaders together to connect with colleagues, share ideas and be recognized for their commitment to excellence. The night celebrated the contributions and achievements of some of the best life sciences leaders.

2017 Industry Growth Winners pictured with GaBio President & CEO Russell Allen.

The evening's top recognition, the Industry Growth Awards, were presented to Tiffany Wilson, the Chief Executive Officer of the Global Center for Medical Innovation and T3 Labs in Atlanta and Steven L. Stice, PhD, the D.W. Brooks Distinguished Professor, GRA Eminent Scholar, Director of the Regenerative Bioscience Center at the University of Georgia, and founder of two Athens-based biotechnology companies, ArunA Biomedical and SciStem. Georgia Bio would like to thank its sponsors, the awards committee and the life sciences community for another successful celebration of the outstanding achievements of the winners in each category.

The event was sponsored by UCB, the presenting sponsor, with additional support from Arbor Pharmaceuticals, Troutman Sanders, Jones Day, VWR, and the Atlanta Science Festival. Photos are available for viewing at www.gabio.org/awards.

GaBio Chair Jay Yadav Addresses Attendees

2017 Award Winners include:

Georgia Bio Industry Growth Award

- Tiffany Wilson, Global Center for Medical Innovation
- Steven L. Stice, Ph.D., University of Georgia

Deals of the Year

- Alimera Sciences
- Arbor Pharmaceuticals, LLC
- Axion BioSystems, Inc.
- Clearside Biomedical, Inc.
- CryoLife, Inc.

Phoenix Award: National Cell Manufacturing Consortium

Innovation Award

- Latha Ganeshan, CEO and Founder, Zywie
- Robert Ivarie Ph.D., B.S., University of Georgia/Synageva
- Matthew Lyon, M.D., Augusta University
- David Munn, M.D., Augusta University

Georgia Bio Community Award

- Rafael V. Andino, Clearside Biomedical
- Derek Eberhart, Ph.D., University of Georgia
- Abel De La Rosa, Ph.D., Drug Innovation Ventures at Emory & The Emory Institute for Drug Development
- Georgia Power
- UGA Complex Carbohydrate Research Center

Lawmaker of the Year: Lieutenant Governor Casey Cagle

Emerging Leader of the Year

- Christine Hang, Flow MedTech, Inc.
- JoAnna Pendergrass, DVM, JPen Communications

Teacher of the Year: Marc Pedersen, Ed.S., Paulding County High School - Dallas, GA

The Top Tech of 2016

Biotech Primer

CAR-T PRIMER

The hottest cancer therapy in the pipeline — chimeric antigen receptor therapy (CAR-T) — continued to mature in 2016 with its first FDA approval for blood cancer patients set to arrive this year.

What's next in the world of CAR-T? A whole lot:

- Additional cancer and autoimmune disease indications in preclinical development.
- Added safety features.
- More affordable "off the shelf" versions of the technology.

Let's first welcome 2017 by reviewing how this key therapeutic works; we will continue our discussion by covering the future of CAR-T in our next edition.

TERM OF THE WEEK: KILLER T-CELLS

CAR-T therapy is modeled after a cell in the immune system known as the killer T-cell. The job of a killer T-cell is exactly what the name implies — to kill dangerous cells. They target diseased cells in the body via their receptors: each one has a uniquely shaped receptor, and will recognize its intended target because the shape of its receptor "matches" or fits into a uniquely shaped surface protein found only on diseased cells. Once the Killer T-cell "docks" onto its target, it injects an enzyme which triggers death. The result: no more bad cells.

WHY CAR-T?

In theory our immune system should recognize the unique proteins presented on all diseased/cancerous cells; however there are two main reasons this doesn't always happen:

1. Early on in the tumor development, the cell composition is similar enough to healthy tissue that it can be overlooked by the immune system.
2. Later as a tumor progresses, it releases chemical signals that suppress the immune response, helping it to evade detection. This trickery is known as the tumor microenvironment and once again the dangerous cancer cells can pass by undetected.

So what's a scientist to do?! Figure out a way to train killer T-cells to ALWAYS recognize and destroy cancer cells... enter CAR-T.

HOW TO TRAIN AN IMMUNE SYSTEM

Killer T-cells are "trained" to go after early and late stage cancer by having their physical structure altered. This alteration is accomplished by fusing an antibody with the receptor of a killer T-cell to create a chimeric molecule — or the "C" in CAR-T.

Training day begins by having killer T-cells drawn out of a patient's body and isolated in the lab. Next, scientists deliver a gene to the T-cells that encodes the chimeric receptor. This receptor consists of two parts:

• A targeting domain: This is the part of the chimeric receptor that will be outside of the T-cell. It is composed of an antibody that will recognize and dock onto a unique surface protein of the patient's cancer.
• An activation domain: This part of the receptor will be triggered once the targeting domain is engaged. It will signal to the killer T-cell to:

1. Stay alive.
2. Make copies of itself.
3. Release signaling molecules called cytokines. Cytokines are chemical signals that activate other white blood cells to join the fight against the tumor.
4. Kill the target cell.

The T-cell/antibody hybrid is now a CAR-T therapeutic. It is then multiplied in the lab and infused back into the patient's body. Once inside, the CAR-T locks onto its cancer target, replicates, sends out cytokines, and kills the designated cancer cells. The CAR-T will continue to replicate and kill any and all cancer cells recognized by the initial antibody component, with the goal of eliminating the disease.

WHAT'S IN A NAME?

Chimeric antigen receptor therapy broken down:

Chimeric: Composed of components from two distinct parts, such as an antibody and a killer T-cell.

Antigen: A protein that is recognized by an antibody, such as a protein on the surface of a tumor cell.

Receptor: A protein that is embedded in a cell membrane and transmits signals to itself in response to being activated, for example a T-cell receptor transmits signals to the T-cell when it docks onto its target.

Therapy: A treatment meant to manage or cure a disease.

BIOTECH TARGETING BLOOD CANCERS

CAR-T therapy has shown great promise in targeting blood cancers such as leukemia and lymphoma. Kite Pharmaceuticals' positive Phase II data for diffuse large B-cell lymphoma has prompted the Santa Monica-based company to file a rolling submission of its biologics licensing application (BLA) for their KTE-C19 CAR-T product. Novartis (Basel, Switzerland) has also been conducting clinical trials of its CTL019 CAR-T product for acute lymphoblastic leukemia, it has plans to submit a BLA in early 2017.

Bio/Med Investor Network Boasts Record Attendance at Q1 2017 Investor Dinner

Stephen MacDonald

The Bio/Med Investor Network (BioMed) held its first investor dinner of 2017 on February 27th. Over 50 BioMed members and their guests were in attendance to hear from presenting companies neoSurgical Limited and Nephrogen Sciences. The members also heard from Rick Coulon, CEO of Accutis Inc., a BioMed portfolio company, who provided a progress report of the company.

Bio/Med Investor Network, Inc. is a community-supported angel network of high net-worth accredited investors who have joined together to evaluate and make individual investments in early-stage biosciences and certain other healthcare companies. The member-driven screening committee selects a limited number of applicants to present to the membership at the quarterly dinners.

"I was very impressed with the turnout and at how well things have evolved with this group. I'm looking forward to future events. The BioMed team is working hard to keep this investment group moving forward." said Ed Schutter, CEO of Arbor Pharmaceuticals and BioMed member.

The Bio/Med Investor Network was formed in 2014 to address the need for funding in early-stage bioscience and healthcare companies. Network members are accredited angel investors specializing in these fields to provide a high level of scrutiny and guidance for growing companies. The Network is based in Atlanta, Georgia with about 50 investor/members.

New to the investor dinner program was a discussion group coined "Angel Talk" preceding the cocktail reception. The Angel Talk session is an informal discussion on angel network issues and investing concepts for members interested in attending. This session was well-received with 28 individuals attending a discussion delivered by BioMed member, John Huntz and Board Chair and BioMed sponsor, Frank McDaniel. "We were surprised and delighted at the turnout for the Angel Talk session last Monday. It demonstrates an interest within our membership for some additional context and information on relevant topics. We look forward to continuing the Angel Talk sessions at future dinners."

BioMed is currently seeking applications from U.S. based early-stage biomedical, medtech and health IT companies. Typical investment stage target is from $200,000 to $2,000,000, but companies outside of these stages of fundraising are also considered. The highly qualified screening committee selects a limited number of applicants for an opportunity to present to members at the quarterly dinners.

"Momentum has been building since the very beginning under the careful stewardship of the team – and the addition of Stephen MacDonald as Managing Director is preparing us for the next level of achievement." said Michael Cassidy, CEO and President of the Georgia Research Alliance and BioMed sponsor.

More information can be found at www.biomedinvestors.com.

GaBio Members Can Start Saving with UPS Today!

BIO Business Solutions®, BIO's cost-savings program, has announced a new partnership with UPS. The UPS Savings Program is available to BIO and Georgia Bio members, and offers a best-In-class, cost-savings program for shipping services, and discounts on specific value added healthcare services and solutions to meet the specialized needs of healthcare and life science companies.

Full program benefits can be found at www.bio.org/bbs/UPS.

UPS understands healthcare logistics and can give you single-source management of transportation and freight shipping throughout the delivery process. UPS offers a range of specialized services for companies in biotechnology, diagnostics, pharmaceutical and medical device manufacturing that are built on a dependable supply chain network, enhanced by 24/7 risk mitigation monitoring tools and gold standard service. Our attitude toward healthcare is summed up by the philosophy: "It's a patient, not a package"®. For more information on UPS's healthcare services, visit http://ups.com/healthcare.

ShareVault Webinar – "Microbiome: Hope or Hype"

Stephen MacDonald

Date & Time: Wednesday, March 29, 2017, 11am-12:15pm Pacific / 2pm-3:15pm Eastern

Moderator: Ravi Kiron, Managing Partner, BioPharm Strategy Advisors

Panelists: Colleen Cutliffe, CEO, Whole Biome
Mohan Iyer, CBO, Second Genome
Mark Wilson, CEO, MartiSys Bioscience

This ShareVault-sponsored web panel discussion with leaders in the microbiome research and development space will look at whether future advances in deciphering this paradigm can provide hope and revolutionize the healthcare industry and help innovate new ways to control disease and human growth, or whether the hype will unlock a Pandora's Box of issues, which we understand very little about now.

The panelists will uncover some of these truths and discuss where this new field of R&D is headed. Here are some of the topics and questions the panel will address:

• Is the real way to a person's heart through their stomach? Maybe the ancients knew!

• Financial approach and ROI for early and late stage investments into this arena

• Timelines for impact in terms of developing therapeutics and diagnostics

• Advances in the sciences needed to "really" understand what's going on

• Where is this field headed in terms of pharma and healthcare involvement?

• Opportunities and challenges of delving into the human microbiome

• What partnerships are critically needed between academia, companies, financial institutions and the government?

• Predictions on translation of early successes to therapeutic areas

• FDA and the microbiome – friend or foe?

Click here to learn more and register: http://bit.ly/2mgClMD

Featured New Member: **Matrix Surgical USA**

Matrix Surgical USA designs, manufactures and distributes OMNIPORE® porous high-density polyethylene implants for craniofacial reconstructive and aesthetic surgery. Manufactured in the USA, OmniPore Surgical Implants meet the highest standards for quality and reliability, and provide surgeons with an expanded range of options.

OMNIPORE implants have been cleared by the FDA, CE marked in Europe and approved by some of the world's most stringent regulatory authorities such as ANVISA (Brazil), KFDA (South Korea), TFDA (Taiwan), TGA (Australia) and many other countries.

Learn more here.

Upcoming Events

Atlanta Science Festival
March 15-25, 2017

BIO-Europe Spring 2017
March 20-22, 2017

SEMDA PitchRounds-Atlanta
March 21, 2017

Genes in Space and on Earth too
March 24, 2017

Take Charge of Your Career- Leadership & Prof Dev. Tips
March 28, 2017

Webinar: The State of Medtech Innovation in SE US
March 28, 2017

RAPS Atl Chapter: SE Region Regulatory Career Day
March 30, 2017

"Power to Influence" - Professional & Leadership Training
March 30, 2017

"Management Essentials" - Professional & Leadership Training
March 31, 2017

BIO Legislative Day Fly-in
April 4-5, 2017

Swings Fore STEM Golf Outing 2017
April 10, 2017

SEMDA 2017 Annual Conference
April 19-21, 2017

World Orphan Drug Congress USA 2017
April 19-21, 2017

UGA GRADS Industry 2017
April 27, 2017

Raise Forum
April 28, 2017

2017 Academic & Industry Intersection Conference
May 10, 2017

2017 Georgia Logistics Summit
May 16-17, 2017

Cell & Gene Exchange 2017
May 22-23, 2017

BIO International Convention
June 19-22, 2017

2017 Georgia Bio Innovation Summit
October 24, 2017

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