

Pharmaceutical Knowledge

Boston Area Chapter

www.ispeboston.org



May 2014, Volume XXIV, No. 3

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<u>President's Message: The Weather is Heating Up and So are Chapter</u> <u>Activities!</u>



Dear Boston Area Chapter Members,

As we move past winter and into spring, 2014 is already another great year for the Boston Area Chapter! With membership and participation increasing across all of our events, we continue as the leading example of innovation within ISPE.

I want to recognize and congratulate UMass Dartmouth, Boston University, and Massachusetts College of Pharmacy and Health Sciences Universitythe three new Student Chapters formed this spring. Thank you to all the volunteers from ISPE and the three colleges/universities whose hard work made this dream a reality. In addition, we have now surpassed our goal and have well over 200 Student Members of the Boston Area Chapter!

I would also like to recognize two leading biotech companies who continue to host our events and offer their services in support of the Boston Area Chapter. This year Genzyme/Sanofi and Biogen Idec have hosted multiple educational programs at their facilities. Biogen Idec hosted two events this month alone! It is because of this partnership with industry (not to mention the extraordinary efforts of our volunteers on the Educational Program Committee) that the Chapter is able to continue to bring exceptional educational events to our Members.

Our effort to bring expanded chapter services to all of New England has officially begun. After several months of trials, the Geographic Outreach (GO) Committee launched the first-ever official simulcast of an educational program to a remote location on April 17. The program, which took place at the Massachusetts Accelerator for Biomanufacturing at UMass Dartmouth, was beamed to the Emerging Technologies and Innovation Center at UMass Lowell where a group of members were able to participate (networking reception included) without making the trip south. This use of technology will allow us to bring future educational programs to our Members throughout New England.

As I do every month, please see below for updates related to our goals for the year:

- Monthly Events As we wrap up the first quarter of 2014, the Chapter has already held 11 events. Attendance is growing at all events and they are regularly selling out. Check out the upcoming schedule for events that interest you.
- Student Chapters As mentioned above, we have added three Student Chapters to the ISPE ranks, bringing our student membership to well over 200. We now have 11 active Student

Chapters:

Boston University Mass College of Pharmacy and Health Sciences Massachusetts Maritime Academy Northeastern University University of Massachusetts-Amherst University of Massachusetts - Dartmouth University of Massachusetts -Lowell University of Rhode Island Tufts University University of New Hampshire Worcester Polytechnic Institute

 Membership Growth - Through sustained services to members, we have seen our membership grow to over 1750 members, far and away the largest ISPE Chapter in the world. Our retention rate is also growing and is now at 85 percent.

As we continue to move forward, there are multiple opportunities for Members to assist and volunteer. In addition to contributing to the Chapter's success, benefits of volunteering include:

arowing your network of professional contacts

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A list of our committees is on the Chapter website; take a look and see where your interest may lie. By joining a committee, you will have a chance to work with some highly motivated people and make connections that will last throughout your career!

Sincerely,

Dan Ramsey

President ISPE Boston Area Chapter

Chapter Bulletin Board

Product Show Booths Selling Fast - Choose Your Booth Location Before It's Too Late

Exhibitors! Registration for the 23rd Annual Boston Chapter Product Show on October 1 is now open and booths are disappearing fast. This year's Show is shaping up to be even bigger and better than last year's which was huge! How huge? Check out the brand-new show video at <u>http://www.ispeboston.org/ProductShow/index.html</u> and see why your company needs to be at this year's Show. Then register and pick your booth location at <u>www.ISPEBoston.org/ProductShow</u> before it's too late. And while you're there, be sure to sign up for one (or more!) of the many new sponsorship and advertising opportunities available this year. See you at Gillette on October 1!

Scholarship Applications Due June 15; Lifetime Cap Raised to \$8,000

The Chapter's Board of Directors recently voted to increase the Joel Goldenberg Memorial Scholarship lifetime cap to \$8,000 per individual, \$4,000 undergrad and \$4,000 grad/continuing ed. Awards of up to \$2,000 are made twice each year, with applications due June 15 and November 15.

To qualify, scholarship applicants must be members of the Boston Area Chapter and submit an

application providing information about their academic program, extra-curricular activities, volunteer involvement with the life sciences industry, and future career plans. To reflect the needs of its membership the Chapter will accept applications from graduating high school seniors, undergraduate students, graduate students, and Members currently working in industry who are continuing their professional advancement through education.

For complete information about the program and to download an application, visit the Chapter website at <u>http://www.ispeboston.org/scholarship</u>. And remember, the next round of applications are due June 15, 2014 so don't delay!

Educational Program Simulcasts Extend Chapter's Reach

In an exciting first, the Chapter has presented an educational program at two venues at once via simulcast. The April 17 program at the Massachusetts Accelerator for Biomanufacturing (MAB) at UMass Dartmouth was beamed to the Emerging Technology and Innovation Center at UMass Lowell where a group of members viewed the entire presentation and panel discussion live on screen. Networking receptions with light dinner refreshments preceded the program at both locations.

Following this successful launch, the Chapter and its Geographic Outreach (GO) Committee hope to make selected educational programs available at population hubs throughout New England. Many thanks go to the Educational Program and GO Committee members, event coordinators and our hosts at both locations who coordinated their efforts to ensure a smooth and seamless experience for all attendees. Look for full details regarding this exciting event in the July newsletter.

CPIP (Certified Pharmaceutical Industry Professional) Program Ends Its Run

After careful deliberation, the ISPE International Board of Directors and upper management have made the difficult decision to terminate the CPIP program based on its high cost. Although the Boston Area Chapter has been extremely successful with its CPIP Study Group program, Chapter leaders were unable to persuade the International Board to reverse their decision. While ISPE will not support new CPIPs, they have committed to allowing those who have attained the valued CPIP credential to maintain it.

ISPE's decision does not in any way diminish the value of the coveted certification and for those Boston Area Chapter Members who participated in the Chapter's CPIP Study Group program, knowledge was gained, industry experience shared and all involved greatly benefited from the unprecedented show of teamwork, mutual support and respect among participants.

Many thanks go to the many Chapter volunteers who spent countless hours developing and implementing the CPIP Study Group program, with special recognition to Joyce Chiu, Brian Hagopian,

Training in Pilot-Scale Lab



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Doyle Johnson and John Spohn. These dedicated Members made the CPIP program an unqualified success at the local level and the Boston Area Chapter the undisputed worldwide leader in CPIPs.

And last, but by no means least, congratulations to all the Boston Area Chapter CPIPs. You've worked hard and earned it!

- Can Aktar, CRB Consulting Engineers
- Johnathan Antonizio, Shire HGT
- Troy Appleton, Foster Delivery Science
- Brent A. Arbogast, Critical Process Filtration
 David Barabani
 - David Barabani
- Michael Beckwith, Commissioning Agents
- James Carmichael, Alexion Pharmaceuticals
- Daniel Carpenito, Commissioning Agents
 Maurizio Cattaneo, BioVolutions
- Michelle Cheslek, Genzyme
- Joyce Chiu, Honeywell Safety Products
- Matthew Coleman, MannKind Corporation
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- Marc Fleischman, Genzyme Corp.
- Samir Gondalia, Pfizer
- Samir Gondalla, Plizer
- Zebulon Jones, Genzyme Corp.
- Brian Hagopian, Clear Water Consulting
- Michael Harrison, Biotechnicians Network
- Daniel Haun, Commissioning Agents
- Heather Hochuli, Pfizer
- Bob Huddy, Commissioning Agents
- David Hyde, Lantheus Medical Imaging
- Paige Kane, Pfizer
- Vijay Kasireddy, Alexion Pharmaceuticals
- Rick Kotosky, Kotosky Consulting
- Robert Lucas, Commissioning Agents
- Allan MacDonald, BosBio
- Scott D. Mackley, Commissioning Agents
- Pamela McGuinness, Eisai Research Institute
- Paul J. Meehan, Jr., AbbVie Bioresearch Center
- Chad E. Michael, Commissioning Agents, Inc.
- Robert Mitchell, Robert Mitchell Engineering
- Jakub Mocny, Superior Controls, Inc.
- Russell J. ("Rusty") Morrison, Commissioning Agents
- Aarash Navabi, Genzyme
- Michael O'Connor, Pfizer
- Alan F. O'Driscoll, Genzyme
- Brian Pochini, Genzyme
- Bob Prytko, Sunovion Pharmaceuticals
- James Quinn, M+W Group
- Jean Quong, Genzyme Corp
- Moises Sabio, DMS Consultants
- Robert Sampson, Pfizer
- Terry Seanard, Jr., New England Controls
- Robert Snow, Genzyme
- John Spohn, Hargrove Life Sciences
- Curtis Steenstra, Pfizer
- Victor Taubinger, Shire HGT
- Andre Walker, Biogen Idec

The Chapter salutes you, one and all!

eNewsletter Ad Space Expanding - Sign Up Now!

Now that the Boston Area Chapter has grown to include over 1,750 members located throughout all of New England, our eNewsletter ad space is also increasing. This gives Chapter Members a new opportunity to join the ranks of eNewsletter advertisers and gain valuable media exposure while helping to support the Chapter's expanding activities.

This additional ad space will disappear quickly so don't waste any time. Visit the sponsorship page on our website at <u>www.ISPEBoston.org/Sponsorship</u> and open the sponsorship application. Choose the eNewsletter option that works best for you - ads are available in two sizes and run for six months or a full year - then pay by credit card online. It's that simple! Or if you'd rather, contact the Chapter office at 781.647.4773 or office@ispeboston.org.

While you're there, be sure to explore the full range of sponsorship options available in addition to eNewsletter ads, including educational programs, social events and website ads. Using the sponsorship application as your worksheet, you can view your savings as you select various combinations of sponsorship options - the more you choose, the bigger your discount!

Don't delay! Visit <u>www.ISPEBoston.org/Sponsorship</u> and add your name to the growing list of sponsors that gain valuable exposure while helping support the Chapter's activities. Have questions? Contact the Chapter office at 781.647.4773 or <u>office@ispeboston.org</u> and we'll be happy to help!



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Want to Become a Chapter Sponsor? It's Easy!

Ever wonder how to become the Sponsor of a Chapter educational program or social activity or how to land one of the coveted eNewsletter or website advertising spots? To answer these questions, the Chapter has created a new website resource at <u>www.ISPEBoston.org/Sponsorship</u> containing all the information you need to know to become a Chapter Sponsor. So don't delay, visit our website and add your name to the growing list of Sponsors who gain valuable exposure while helping the Chapter better serve its Members.

Upcoming Chapter Events - Mark Your Calendar

Thursday, May 8, 2014

Young Professionals: Bowling Social The Flatbread Company, Somerville, MA

Come join the ISPE Boston Area Chapter Young Professionals at The Flatbread Company in Davis Square for an evening of pizza, socializing, and candlepin bowling at the famous Sacco's Bowl Haven! Enjoy a variety of wood-fired, clay oven flatbread pizzas (vegetarian options will be available). Bowling shoes will be provided and all attendees will have access to a cash bar.

Register Today:

http://www.ispeboston.org/eventcal/calendar.html?action=display_event&oid=441

Thursday, May 15, 2014

Upstream Processing: Development and Optimization WPI, Worcester, MA

The successful implementation of microbial and animal cell culture processes requires optimization of a number of variables. A clear understanding of cell line development, media optimization and the control of critical growth parameters assures that upstream scientists and technicians are capable and confident in their handling of these processes. This dual track program focuses on the principals of cell growth and the unit operations that facilitate the manufacturing process.

Register Today: http://www.ispeboston.org/eventcal/calendar.html?action=display_event&oid=401

Thursday, May 29, 2014

Young Professional Red Sox Social Fenway Park, Boston, MA

Come join the ISPE Boston Area Chapter Young Professionals for a fun-filled Red Sox game against the Atlanta Braves on Thursday, May 29th. We'll meet to pick up tickets and enjoy some social pregame beverages at Baseball Tavern. Availability is limited, so register today!

Register Today: http://www.ispeboston.org/eventcal/calendar.html?action=display_event&oid=440

Sneak Preview of Upcoming Events

Sunday, June 15, 2014 Scholarships Due

Thursday, June 19, 2014 C&Q as a Risk Management Strategy

Monday, August 18, 2014 Summer Golf Tournament

Members Travel to Devens for February Program

by Mike Denault, Denault Associates

The Boston Area Chapter conducted an educational program on February 20 at the Johnson Matthey Pharma Services facility in Devens, Massachusetts consisting of two presentations, both of which were based on half-day training workshops held at the FDA headquarters in Rockville, Maryland.

The first presentation was given by Alfredo Canhoto, Associate Director of Technical Solutions for the ProPharma Group. The presentation, entitled "Process Validation Guide, Regulatory Expectations and Best Practices," covered the most recent FDA regulations and guidance on Process Development/Design, Process Validation and Process Performance Qualification as well as Continued Process Verification. The presentation traced the evolution of the FDA's regulations and guidance on validation from 1987 to 2011, from a single, explicit event to a risk-based, lifecycle process. Discussed at length were the current FDA expectations for Process Validation to be a series of activities occurring over the lifecycle of a product from Process Design through Process Qualification and Continued Process Verification. The FDA's expectation of more process understanding and development of the control strategy earlier in the lifecycle and the use of risk based assessments and statistics to evaluate variability were discussed.

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The following part of the presentation covered how to utilize the information and assessments from this first phase of the lifecycle to develop and execute the Qualification Phase. Discussed next was how to establish a system for Continued Process Verification to assure that the process remains in control. The FDA's expectations as well as best practices from industry were covered. The EU Continuous Process Validation approach was also presented.

Mike Byrd, Director of Computer Systems Validation at the ProPharma Group, gave the second talk on Computer Systems Validation. This presentation covered the Fundamentals of Computer Systems Validation, Considerations for Data Integrity, Requirements for Electronic Batch Records and concluded with a discussion on Emerging Technologies. The fundamentals portion included a review of the FDA's current guidance documents as well as CFR 21 Part 11. The objectives of Computer System Validation and Life Cycle Approaches as well as GAMP 5 and ASTM E2500 Risk Assessments were discussed.

An overview of the FDA's current interpretation of Part 11 that limits the number of records that are subject to Part 11 requirements was presented. Requirements for GLP's were also discussed. The Data Integrity portion of the talk covered sources of variation in information management as well as accidental data loss and corruption and intentional data falsification. SOP's, system testing and verification, backup and recovery procedures as well as disaster recovery and business continuity and periodic reviews were covered. The requirements for Electronic Batch Records were discussed as well as an overview of Manufacturing System Architectures. An overview of emerging technologies and their challenges was presented. A discussion of virtualization, cloud computing, mobile and personal devices and apps and the challenges of asset control, OS and configuration management and control, network connectivity and authentication were also presented.

The Boston Area Chapter would like to thank the speakers for their informative presentations, event sponsor Tufts Gordon Institute for supporting the event and Johnson Matthey Pharma Services for hosting the Chapter at their Devens facility.

Record Breaking Turnout at March Ski Outing

by Jim Grunwald, Commissioning Agents, with photos by Joyce Chiu, CPIP, Honeywell Safety Products

The ISPE Social Committee, supported by a great group of sponsors and a record-breaking number of attendees, provided a fantastic event on March 7 as Chapter Members travelled to Waterville Valley, NH for a day of skiing and networking under blue skies, with great snow conditions.

This year the Social Committee put together a full program and introduced a dinner buffet to feed the

troops after a solid day on the hill, a good move to be sure. An unscripted appearance by ISPE's strongest rapper drove the crowd crazy and asking for a full-fledged set but the mysterious artist held firm, not wanting to over expose his material and budding talent. The crowd was buzzing and a local critic commented that this baritone must have had an operatic background based on his physical presence and sheer volume of sound. We can only hope for an encore at another local event, although it may not be a planned thing.



Rob Mitchell and son Chris enjoy the bus trip north.

Please watch for our spring and summer Golf Outings: April 28 and August 18. In addition, the Social Committee is conjuring up a Spring Social with perhaps more grassroots entertainment from our talented membership.

In closing, the Social Committee would like to acknowledge our fantastic sponsors for all that they do: A/Z Corporation, BR+A, Commissioning Agents, Gilbane, GxP Automation, Integra, Perkins+Will, RW Sullivan Engineers, Safety Partners, Sentrol, Superior Controls, Tobin Scientific & Ultrafiltronics.





Mother Nature awarded the Chapter a day of perfect weather & unbeatable snow conditions.



Paul Doherty (I) and friends take a break between runs.



Chapter President Dan Ramsey (I) and Product Show Committee Co-chair Sean Burgess relax after a long day on the slopes

"Bioprocessing Challenges" Program Enlightens and Engages

by Howard Sneider, CRB Consulting Engineers

On March 27, a group of almost 80 attendees filled the Bio 6 Auditorium at Biogen Idec to participate in a technical seminar discussion entitled, "Bioprocessing Challenges: High-Titer Mammalian-Based Cell Systems" with guest speaker Jonathan Romero Ph.D., Associate Director of Manufacturing Process Engineering at Biogen Idec.

The presentation began with an analysis of commercial processes for monoclonal antibody (mAB) production. Existing facilities were designed for the lower cell concentrations and titers of the previous generation and current crop of mAB drugs. Jon demonstrated that predictions of future cell

concentrations and titers will bottleneck current cell culture clarification and purification processes. In addition, rapid production of large volumes of drug substance may require a large investment in

cryostorage. Jon elaborated on the economics of high-titer production. He illustrated that, as production titers increase, economies of scale lower production cost per gram but the cost of goods supplied (COGs) is dominated by purification-related expenses.



Biogen Idec's Jonathan Romero drew a record crowd to Kendall Square.

Finally, we circled back to the capability of today's typical mAB process. Jon demonstrated that our current reliance on protein A capture chromatography may be challenged as titer increases, leading to unacceptable levels of column cycling and buffer volumes. Other sections of the plant may be idle while purifying, creating a condition of low operational effectiveness. Jon suggests that protein precipitation may be used to either eliminate protein A capture or decouple the downstream purification process from cell culture harvest.

In the first example, precipitation provides adequate purification without requiring protein A and possibly without requiring additional capture chromatography steps. This process has a lower COGs than the current platform process and may potentially use less buffer volume. In the second example, precipitation removes sufficient Host Cell Protein (HCP) and DNA for the temporary storage of inprocess material, which is later thawed for Protein A capture. This decoupling of the purification process from harvest means that a fraction of the cell culture harvest, and therefore a reduced titer, may be purified according to today's methods. A third example was also presented where in-process material is frozen after precipitation and purified without protein A, thereby achieving the benefits of a low COGs with high operational effectiveness.

Throughout the discussion Jon surveyed the audience and paused for questions, which added a lively dimension to the proceedings. Participants clearly enjoyed the program and were impressed by the high quality of the information presented.

On behalf of the entire Boston Area Chapter, the program manager would like to thank our host, Biogen Idec; our program sponsors, CRB Consulting Engineers and Tufts Gordon Institute; and Dr. Jonathan Romero who provided a thoroughly interesting and educational seminar.

Young Professionals Corner: Attention Future Leaders - No Need to Wait!

by David Goldsmith, Medix Scientific

The ISPE Boston Area Chapter recently held the latest edition of the Young Professional Series of educational programs entitled "Engineering Leadership and How to Influence Without Authority." This event was held at Genzyme's worldwide headquarters in Cambridge on April 8 and attracted a wide array of attendees with a variety of backgrounds.

Jared Marshall of Genzyme along with Andrea Massa of Burkert Fluid Control Systems, both members of the Chapter's Young Professionals Committee, welcomed the attendees following a quick

reception in the lobby. The Young Professionals Committee was joined by Professors Sam Liggero and Mary Viola, each of whom brought extensive experience in the industry as well as leading Tufts' Gordon Institute Master's Program in Engineering Leadership.

Dr. Liggero, a key member of Polaroid prior to joining the Gordon Institute, provided insight into organizational leadership and how to lead and motivate when the work environment is constantly changing. As our industry can change at a moment's notice, providing leadership and motivation in dire times is crucial to the development of an organization. As a leader, Dr. Liggero empowered his team to provide incentives such as spot recognition using funds allocated to buy a pizza or fund a happy hour. Opportunities for motivation can be amplified by keeping the team in tune with the state of the business and highlighting the advances the company is making, as well as providing continued knowledge development for employees so they "don't stay stale." Dr. Liggero drove home the point that "the way you get things done is through people." In other words, invest in those around you to succeed as a leader and an organization.

Dr. Mary Viola seamlessly followed up by appealing to the crowd, mainly young professionals, with tips on how to "influence without authority." She introduced the notion that you can be a leader without a being a manager and there are some tactics that can be used to step into that role without having direct reports. Dr. Viola stressed the importance of reciprocity along with using soft tactics over hard tactics. Working with someone to move towards a goal and providing a "give and take" to accomplish a goal when under pressure can provide opportunities to be a leader in a peer-relationship.

She explained that there are numerous "currencies of exchange," such as understanding what your

individual teammates are passionate about and how they are rewarded. Everyone is inspired by different things, so find out what will help you get your co-workers motivated to accomplish the task. Lastly, Dr. Viola provided insight into how to deal with that co-worker you may see as the "enemy." Your body language and speech patterns will show this in your communication. If you are able to view them as the "ally," visualizing working as a team to accomplish your goal, it will shift the paradigm in both your minds moving forward.

Finally, Dr. Liggero and Dr. Viola invited three Gordon Institute alumni to the floor to answer questions from the crowd. Kyle McElearney of Biogen Idec and Michael Cody and Mary Pirone of Genzyme provided insight into how they have taken what they learned and applied it in their jobs. Messages such as being happy and enjoying your job, along with basing your communication on the temperature of the room and the culture of the organization are vital in your ability to be viewed as a leader.

Following the wrap up, the attendees gathered down the street at Mead Hall to discuss what they learned and share experiences. While many of the attendees came from different roles in organizations of different sizes, there was one over-arching theme: the ability to lead through tough times does not only come from a managerial role but can be accomplished through effective and well-thought out communication.

The Boston Area Chapter and Young Professionals Committee would like to thank the Tufts Gordon Institute, Genzyme, Mead Hall and our speakers for helping make this a successful event in the Young Professional Series. Please stay tuned to ISPE publications to keep up-to-date with the many Chapter and YP events taking place in the Greater Boston area during the upcoming months. We look forward to seeing you there!

Spotlight Interview with Anatoliy Tereshchuk, Joel Goldenberg Memorial Scholarship Winner

The Boston Area Chapter congratulates Anatoliy Tereshchuk, a twotime Joel Goldenberg Memorial Scholarship award winner. Anatoliy is an UMass Amherst chemical engineering major with career aspirations in the field of biopharmaceuticals. He is also an enthusiastic ISPE Student Member and is active in his community



where he volunteers in many church activities and supports those in need through the Springfield Rescue Mission. Anatoliy happily agreed to answer a few questions for Chapter Board Member Jillian Willard:

You were recently awarded the Boston Area Chapter Joel Goldenberg Memorial Scholarship. How did you hear about it and what will you be using it for?

I found the scholarship on ISPE's website. I am using the award to fund my education, specifically my biochemistry class. I am currently a junior at UMass Amherst majoring in Chemical Engineering and have maintained a 4.0 GPA throughout my first two years at UMass.

Why did you decide to join ISPE?

I joined ISPE because I am interested in the biopharmaceutical aspect of chemical engineering. ISPE organizes tours of biopharmaceutical companies and holds informative seminars. It also provided me with great opportunities to increase my professional connections.

· What attracted you to the biotech/pharma field?

I am interested in the biotech/pharma field because I enjoy learning about biology and biochemistry. I went to an inspirational company information session hosted by Genzyme about rare diseases, where they shared how their medicine treats people. To be able to work in a field that I am passionate about while having my work help people is truly the most meaningful career choice I can make.

• What ISPE activities have you participated in? What was your favorite?

I attended the 2013 Annual Product Show, which was my favorite. I also attended several seminars that ISPE hosted, such as: Biologics Development and Manufacturing, Engineering at AbbVie and the Basics of Biopharmaceuticals. I toured AbbVie's Biopharmaceutical Manufacturing Facility, Cell Culture Lab, Purification Lab and Scale-Up Lab and the WPI Bioengineering Center.

• Where do you see yourself in 5-10 years?

In 5-10 years I see myself working in a biopharmaceutical company. While working, I plan to earn an MBA to broaden my knowledge of how companies are managed.

New Student Chapters Forming at Record Pace

by Brian Hagopian, Clear Water Consulting, with photo by Mark Jodoin, Symbotic

Welcome to our Newest Student Chapters! The Chapter is pleased to welcome students from our two newest Student Chapters at Boston University and UMass Dartmouth. BU and UMD join the

Massachusetts College of Pharmacy and Health Sciences University - as the three brand-new Student Chapters formed this spring!

Student membership and participation at ISPE events is at an all-time high. If you've attended an educational program recently, you've probably noticed more young faces in the audience. It's all part of the Chapter's plan to involve students in the local life sciences industry and it's working! Next time you attend an ISPE event, please seek out students in attendance and make them feel welcome. You would be absolutely amazed at what a small gesture will do (and, after all, they may be working for you someday)!



Poster Contest judges Chris Opolski, Janet Tice runner-up Mo Zhang, winner Nidhi Maniar (center) and judge Rick Pierro shown at the Annual Student Poster Contest.

Biogen Idec Plant Tour Boston Area Chapter Student Members got a rare opportunity to take a peek inside a biotech manufacturing suite during our March 25 tour at Biogen Idec in Kendall Square which was organized and coordinated by Biogen Idec engineer and Chapter Director Tom Choyce. Students were primed on what they were about to see by Alex Cardoso, Biogen Idec Senior Engineer, who gave a great "Biotech 101" talk focused on downstream processing and the facilities required to support these operations.

Students chose between touring the manufacturing suite or the critical utilities that support manufacturing operations. Biogen's tour guides gave the students great insights during the tours and everyone walked away feeling like they got something special at this event!

Job, Co-op, and Internship Postings The Chapter continues to post entry-level positions, student internships, and co-op positions in the Student Development section of the Chapter website to help our Student Members find positions in the industry. That's right, jobs and lots of them! Look for this area of our website to populate quickly and grow as positions from major area life science companies as well as startups become available to us.

Annual Student Poster Competition The Chapter held its Annual Student Poster Competition on April 17 at the brand-new Emerging Technology and Innovation Center at UMass Lowell. For the first time, the Chapter held the Poster Contest in conjunction with an educational program, giving our students the opportunity to meet and network with local industry professionals. Look for this to continue in future years. In another first, the Chapter awarded \$500 to the winning student. This year's graduate category winner was Nidhi Maniar from Northeastern who presented her research on metabolic pathways of radioactively labeled alcohol in mice. Congratulations to Nidhi who will be representing the Chapter at the International Poster Competition being held at the ISPE Annual Meeting in Las Vegas, October 12-14, 2014.

ISPE's Message is Spreading! Word is really starting to spread across local campuses. Student membership is at an all-time high at our existing Student Chapters but the best news is that new Student Chapters have been forming at a record pace! We're staying active and are in early discussions with RPI, UConn and MIT so look for developments on those fronts in the future! All this activity takes a concerted effort and lots of energy. If you're interested in helping out, just shoot us an email. Whether you have an hour a year or an hour a month, we could sure use the help. Trust me, this is a decision I guarantee you won't regret!

Students Attend ISPE Educational Events For Free! Remember, once you join ISPE as a Student

Member (<u>http://www.ispe.org/membership</u>), you will be able to attend all Chapter educational and related events for free! Membership in the Boston Area Chapter truly does have its privileges!

Industry News in Brief

by Jillian Willard, Genzyme, a Sanofi Company

Industry News In Brief, a regular feature of the Boston Area Chapter Newsletter, presents news items concerning companies in the pharma, biotech, medical device and related fields, with an emphasis on companies with a local presence and topics of special interest to our readers.

FDA Approves Vertex Drug for Cystic Fibrosis

Vertex Pharmaceuticals announced that the FDA approved a supplemental New Drug Application (sNDA) for Kalydeco for people with cystic fibrosis (CF) ages 6 and older who have one of eight additional mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. In the United States, approximately 150 people ages 6 and older have one of the additional eight mutations for which Kalydeco is now approved. Vertex has also filed regulatory submissions in Europe, Canada and Australia for approval of Kalydeco in additional people with CF ages 6 and older. In Europe and Australia, approximately 250 people with CF have these additional mutations.

CF is caused by defective or missing CFTR proteins that result from mutations in the CFTR gene. The defective function or absence of CFTR proteins in people with CF results in poor flow of salt and water into and out of the cell in a number of organs, including the lungs. Kalydeco facilitates increased chloride transport by potentiating the channel-open probability (or gating) of the CFTR protein. (Source: Vertex Website, 14 February, 2014)

Biogen Idec and Whitehead Institute to Collaborate

The Whitehead Institute announced it has entered into a scientific research collaboration with Biogen ldec aimed at driving early stage research that may lead to the development of novel therapies across a broad range of disease areas.

The agreement calls for Biogen Idec to provide \$5.25 million over the next three years to support research projects Ied by Whitehead Institute principal investigators. A joint committee of Whitehead and Biogen Idec scientists will determine the research projects to be funded under the agreement. These pioneering programs will pair Whitehead Institute researchers with counterparts from Biogen Idec and will focus on improving human health through basic biomedical research in the areas of immunology, neurology, developmental biology, genetics and genomics.

The Whitehead Institute is a non-profit research institution dedicated to improving human health through basic biomedical research. Wholly independent in its governance, finances, and research programs, Whitehead shares a close affiliation with Massachusetts Institute of Technology through its faculty, who hold joint MIT appointments. (Source: Whitehead Institute Website, 25 March, 2014)

EMD Serono Enters into Research Agreement with Pfizer and Broad Institute

EMD Serono, a subsidiary of Merck KGaA of Darmstadt, Germany, announced they have signed a research agreement with Pfizer and the Broad Institute in Cambridge. The research project will be jointly funded by EMD Serono and Pfizer. The collaboration is focused on the genomic profiling of Systemic Lupus Erythematosus (SLE) and Lupus Nephritis (LN) patients. SLE is a systemic autoimmune disease, and can cause LN, an inflammation of the kidney. In SLE patients, in addition to the kidney, other tissues and organs can be affected, including the skin, the nervous system, or joints.

As part of the collaboration, the Broad Institute will investigate clinical samples obtained from SLE and LN patients, applying biochemical and next-generation sequencing technologies. They will also analyze immune cell subpopulations. The goal is to identify biomarkers to better define target patient

populations for future therapies. In addition, through computational modeling approaches, the project aims to identify key molecular drivers of SLE and LN kidney flares, and thereby to discover potential novel drug targets as the basis for innovative therapies.

Under the terms of the agreement, EMD Serono and Pfizer, as sponsoring members, will receive realtime access to all data and analysis. In addition, both companies will have the ability to send a research scientist to the Broad Institute to foster exchange of technology expertise in the area of computational and experimental genomic profiling. (Source: EMD Serono Website, 01 April, 2014)

FDA Advisory Committee Recommends Approval of MannKind's Drug for Diabetes

MannKind Corporation announced that the Endocrinologic and FDA's Metabolic Drugs Advisory Committee voted 13 to 1 to recommend that Afrezza (insulin human [rDNA origin]) Inhalation Powder be granted marketing approval by the FDA to improve glycemic control in adults with type 1 diabetes and voted 14 to 0 to recommend that Afrezza be granted marketing approval by the FDA to improve glycemic control in adults with type 2 diabetes. If approved, Afrezza would be the first ultra-rapid-acting mealtime insulin therapy available in the US.

The FDA is not bound by the Advisory Committee's recommendation but considers its guidance in reviewing the New Drug Application (NDA) that was submitted for Afrezza. The Prescription Drug User Fee Act (PDUFA) date for the FDA to complete its review of Afrezza is April 15, 2014. (Source: MannKind Corporation Website, 01 April, 2014)

Alexion Pharmaceuticals and Moderna Therapeutics Ink Agreement on mRNA Therapeutics for Rare Diseases

Alexion Pharmaceuticals and Moderna Therapeutics announced an exclusive strategic agreement for the discovery and development of messenger RNA Therapeutics to treat rare diseases. Alexion develops and commercializes breakthrough therapies for patients with severe and life- threatening rare diseases. Moderna develops messenger RNA (mRNA) Therapeutics, a treatment which allows for in vivo production of therapeutic proteins. The mRNA Therapeutics platform has the potential to speed the development and manufacture of treatments for many rare diseases that are currently untreatable with existing technologies.

Under the agreement, Alexion will make an upfront payment to Moderna of \$100 million to purchase 10 product options to develop and commercialize treatments for rare diseases with Moderna's mRNA Therapeutics platform. Alexion will lead the discovery, development and commercialization of the treatments produced through this broad, long-term strategic agreement, while Moderna will retain responsibility for the design and manufacture of the messenger RNA against selected targets. Following option exercise by Alexion, Moderna will be entitled to drug development and commercial milestone payments, as well as high single to double digit royalties on commercial sales. In addition, Alexion has made a \$25 million preferred equity investment into Moderna.

Moderna has more than 250 patent applications covering novel nucleotide chemistries and drug compositions. The company plans to develop and commercialize its innovative mRNA drugs through a combination of strategic relationships as well as new formed ventures. Founded in late 2010 by Flagship VentureLabs, Cambridge-based Moderna is privately held and currently has strategic agreements with AstraZeneca and Alexion Pharmaceuticals. (Source: Moderna Therapeutics Website, 13 January, 2014)

Baxter to Split into Two Separate Healthcare Companies

Baxter International announced plans to create two separate, independent global healthcare companies - one focused on developing and marketing innovative biopharmaceuticals and the other on life-saving medical products.

The biopharmaceuticals business, with 2013 annual revenues of approximately \$6 billion, consists of a portfolio of recombinant and plasma-based proteins to treat hemophilia and other bleeding disorders, and plasma-based therapies to treat immune deficiencies, alpha-1 antitrypsin deficiency.

burns and shock, and other chronic and acute blood-related conditions. The medical products business, with 2013 annual sales of more than \$9 billion, offers a portfolio of intravenous (IV) solutions and nutritional therapies, drug delivery systems and administration sets, premixed and other injectable drugs, as well as inhalation anesthetics and hospital-based biosurgery products. This business is also integrating the recent acquisition of Gambro AB acquisition. The corporate headquarters of both companies will be located in northern Illinois.

The transaction is intended to take the form of a tax-free distribution to Baxter shareholders of a new publicly traded stock in the new biopharmaceuticals company. The transaction is expected to be completed by mid-year 2015, subject to market, regulatory and certain other conditions. Baxter expects to incur one-time charges related to the transaction during the reporting periods preceding the separation and does not otherwise expect this to impact the company's financial guidance for 2014. (Source: Baxter Website, 27 March, 2014)

Atlas Venture and Biogen Idec Commit to \$17M Funding for Ataxion

Ataxion, Inc, a discovery-stage biopharmaceutical company developing novel therapies for rare, debilitating, and underserved neurologic diseases, has secured Series A financing commitments totaling \$17M from Atlas Venture and Biogen Idec. Atlas Venture is an early-stage venture capital firm that invests in technology and life sciences companies. Ataxion was founded in April 2013 out of the Atlas Venture seed program by Dr. Josh Resnick and Atlas Venture Development Corporation Managing Director and Ataxion Board Member David Grayzel, MD.

Ataxion's lead program is focused on a group of orphan genetic disorders termed hereditary ataxias. These diseases are characterized by dysfunction or degeneration of the cerebellum - the brain's coordination center. Patients with these conditions develop severe difficulties walking, speaking, and performing daily activities. The Ataxion drugs being developed for genetic forms of ataxia may also have the potential to treat other forms ataxia, such as those associated with degenerative neurologic disorders including Multiple Sclerosis and Huntington's Disease.

In addition to participating in the Series A investment, Biogen Idec is providing non-dilutive R&D and other funding to Ataxion. Biogen Idec will have the option to acquire Ataxion to continue development of the program upon completion of the Phase 1 multiple ascending dose (MAD) study at pre-negotiated terms, including upfront & milestone payments. (Source: Saniona Website, 17 March, 2014)

Local Colleges Receive \$1M in Grants to Support Life Sciences Related Capital Projects

Governor Deval Patrick announced nearly \$1 million in grants to support life sciences related capital projects for Quinsigamond Community College (QCC) and Mount Wachusett Community College (MWCC). The \$499,880 grant awarded to QCC will help to fit, furnish and equip specialized space for life sciences programs in its new 30,000 square foot Quinsigamond Engineering, Science and Technology Building located on the main campus in Worcester, slated to open in 2016. The new building and equipment will be used to address the area's growing need for skilled workers in biotechnology biomedical engineering, pharmaceuticals and related fields. Its three floors will house

classrooms, offices and specialized laboratories. The \$20 million facility is a long overdue improvement to existing labs that were constructed in the 1960s.

MWCC was awarded a \$500,000 grant to upgrade aging and outdated equipment and add new equipment that aligns with current industry standards for its core life sciences courses in biology, organic chemistry, inorganic chemistry, biochemistry, microbiology and plant science. These courses provide the basic foundation for MWCC'S existing biotechnology, natural resources and clinical laboratory science degree programs and three new proposed degree programs in quality and analytical technology, liberal arts and sciences biology and chemistry to be rolled out in fall 2014. (Source: Commonwealth of Massachusetts Website, 06 March, 2014)

Prothelia and University of Nevada, Reno Enter Into Agreements with Alexion

Prothelia Incorporated, based in Milford, and University of Nevada, Reno announce that they have entered into strategic agreements with Alexion for the development of Laminin-111, a patented experimental protein replacement therapy for merosin-deficient congenital muscular dystrophy (MDC1A), a life-threatening, ultra-rare disease caused by a genetic deficiency of the Laminin-211 protein.

The three parties have entered into a binding agreement wherein Alexion has an exclusive option to acquire privately-held Prothelia and license Laminin-111 directly from the University of Nevada, Reno upon the achievement of specified research and development milestones. In addition, the University of Nevada, Reno and Alexion have entered into a sponsored research agreement to accelerate further research on the investigational therapy conditioned on the outcome of certain development research to be performed by Alexion.

Laminin-211 provides the necessary structural integrity to muscles, and patients with MDC1A present with respiratory insufficiency, poor muscle tone, muscle atrophy, delayed or absent motor milestones, feeding difficulties, scoliosis and joint contractures. Currently, there are no approved therapies for MDC1A. Laminin-111 is a form of laminin found in embryonic skeletal muscle and has been shown in early animal studies to substitute for the loss of laminin-211. The patented Laminin-111 therapy is based on work by University of Nevada School of Medicine Professor, Dr. Dean Burkin, and was licensed to Prothelia in 2008. (Source: University of Nevada, Reno Website, 13 February, 2014)

Allegro Diagnostics Trial Meets Primary Endpoint

Allegro Diagnostics announced that the Aegis-2 clinical trial has met its primary endpoint, demonstrating that the BronchoGen genomic test improves the accuracy of lung cancer diagnosis when used in combination with bronchoscopy. BronchoGen is Allegro Diagnostics' lead genomic test. Endpoints in the clinical trial include the sensitivity, specificity and negative predictive value of BronchoGen for identifying patients with malignancy. Complete results from the clinical trial will be submitted for publication in a peer-reviewed journal.

Allegro Diagnostics' molecular testing platform utilizes gene expression of normal epithelial cells in the respiratory tract to detect early signs of lung cancer and diseases of the airway. The "field of injury" principle on which the platform is based refers to the detectable molecular changes that occur throughout the respiratory tract in response to smoking and are correlated with disease. These changes can be detected in a gene expression signature from cytologically normal airway cells and indicate the presence of malignancy or disease processes remotely in the lung. Lung cancer tests based on sampling from the bronchus (BronchoGen) and from the nasal passage (NasoGen) have resulted from Allegro Diagnostics' platform and are currently under development. (Source: Allegro Diagnostics Website, 02 April, 2014)

FDA Advisory Committee Recommends Approval of Cubist's Drug for Skin Infections

Cubist Pharmaceuticals announced that the FDA's Anti-Infective Drugs Advisory Committee (AIDAC) voted to recommend approval of Cubist's investigational antibiotic Sivextro (tedizolid phosphate). In the unanimous 14-0 decision, the AIDAC found that substantial evidence of the safety and effectiveness of Sivextro for the treatment of acute bacterial skin and skin structure infections (ABSSSI) was provided. The AIDAC recommendation is not binding on the FDA, but will help inform the FDA as it completes its Priority Review of the NDA for Sivextro, which has an assigned action date of June 20, 2014.

Sivextro is a once daily oxazolidinone being developed for both intravenous and oral administration for the treatment of serious infections caused by certain Gram-positive bacteria, including those caused by methicillin-resistant Staphylococcus aureus (MRSA). The Company's New Drug Application (NDA) submission to the FDA for SIVEXTRO is based on positive data from two global Phase 3 clinical studies, which met the primary and secondary endpoints defined by the FDA and European Medicines Agency (EMA).

Additionally, the EMA recently accepted for review the Company's Marketing Authorization Application (MAA) for the investigational antibiotic, for which Cubist is seeking approval for the treatment of complicated skin and soft tissue infections (cSSTI). A decision from the European Commission (EC) is expected during the first half of 2015.

Cubist specializes in the R&D of antibiotics to treat serious and life-threatening infections caused by a broad range of increasingly resistant bacteria. The Company hopes to deliver at least four new

antibiotics in support of the Infectious Diseases Society of America (IDSA) goal of 10 new antibiotics by 2020. Cubist expects to invest approximately \$400M in 2014 on antibacterial R&D and approximately 75 percent of its employee base is focused on the research, development, commercialization and support of antibiotics. (Source: Cubist Website, 31 March, 2014)

Alnylam Makes Progress on Gene Therapy in Europe

Anylam Pharmaceuticals, a Cambridge company that has allied with Genzyme to develop RNAi therapeutics across the world, has received a positive opinion from the European Medicines Agency Committee for Orphan Medicinal Products on its application for orphan drug status for ALN-TTRsc, a drug developed to treat a rare genetic disease that causes fatal organ and nerve damage. This designation provides drug companies with financial and regulatory incentives to develop and market therapies that treat rare, life-threatening and debilitating diseases affecting no more than five in 10,000 people in the European Union. It includes marketing exclusivity for 10 years after product approval.

Anylam is still conducting clinical trials for ALN-TTRsc, which employs RNAi (RNA interference) gene therapy to treat the degenerative disease known as transthyretin-mediated amyloidosis (ATTR). The company expects to present data from its Phase 2 clinical trial in late 2014.

In January, Alnylam and Genzyme formed an alliance to accelerate and expand development of RNAi therapeutics, with Alnylam retaining product rights in North America and Western Europe and Genzyme obtaining rights to commercialize three Alnylam products. (Source: Alnylam Website, 02 April, 2014)

Tetraphase Pharmaceuticals Awarded Fast Track Status for Antibiotic

Watertown-based Tetraphase Pharmaceuticals announced that the FDA has granted Fast Track designations for both the intravenous (IV) and oral formulations of the company's lead antibiotic candidate, eravacycline. Tetraphase is investigating the safety and efficacy of eravacycline in its ongoing Phase 3 global clinical program. The study is evaluating the IV formulation of eravacycline for the treatment of complicated intra-abdominal infections (cIAI) and eravacycline IV-to-oral step-down therapy for the treatment of complicated urinary tract infections (cUTI).

Fast Track designation is awarded to expedite the study and regulatory review of drugs intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs. Eravacycline became eligible for Fast Track status as a result of being designated a Qualified Infectious Disease Product (QIDP); the QIDP designation also makes eravacycline eligible for priority review and an additional five years of U.S. market exclusivity, if approved.

Tetraphase is a clinical-stage biopharm using its proprietary chemistry technology to create novel antibiotics for serious and life-threatening multidrug-resistant (MDR) bacterial infections, including many of the MDR Gram-negative bacteria highlighted as urgent public health threats by the Centers for Disease Control and Prevention (CDC). Tetraphase has created more than 3,000 novel tetracycline analogs using its technology platform; in addition to eravacycline, Tetraphase has generated multiple preclinical antibiotic candidates that are currently being evaluated for clinical suitability. (Source: Tetraphase Pharmaceuticals Website, 02 April, 2014)

Akebia Therapeutics Raises \$106.9 Million in IPO

Cambridge-based Akebia Therapeutics, a biopharmaceutical company focused on the development of novel, proprietary therapeutics based on hypoxia-inducible factor biology and the commercialization of these products for patients with kidney disease, announced the closing of its initial public offering of 6,762,000 shares of common stock at an initial public offering price of \$17.00 per share. This includes the exercise in full by the underwriters of their option to purchase 879,647 shares of common stock. The aggregate net proceeds to Akebia, after underwriting discounts and commissions and estimated offering expenses, are approximately \$106.9 million. All of the shares of common stock were offered by Akebia.

Akebia Therapeutics is a biopharmaceutical company focused on the development of novel, proprietary therapeutics based on hypoxia-inducible factor biology and the commercialization of these products for patients with kidney disease. Akebia's lead product candidate, AKB-6548, is in a Phase 2b clinical trial in patients with anemia secondary to chronic kidney disease who are not dependent on dialysis. AKB-6548 is being developed as a once-daily oral therapy which works through the inhibition of hypoxia-inducible factor prolyl hydroxylase, leading to stabilization and increased levels of HIFα and therefore improved production of hemoglobin and red blood cells. (Source: Akebia Therapeutics Website, 26 March, 2014)

Novartis Acquires Costim, Expands Cancer Immunotherapy Research Program

Novartis announced that it is broadening its cancer immunotherapy research program with the acquisition of CoStim Pharmaceuticals, a Cambridge-based, privately held biotech company focused on harnessing the immune system to eliminate immune-blocking signals from cancer.

Increasing evidence points to the role of the immune system in controlling cancer and to opportunities for creating effective oncology therapies for cancer patients by stimulating a targeted immune response. Already leading in cancer immunotherapy, with investigative chimeric antigen receptor (CAR) technology being developed in collaboration with the University of Pennsylvania, with this acquisition Novartis is adding late discovery stage immunotherapy programs directed to several targets, including PD-1. These medicines could benefit patients by circumventing cancer's ability to develop resistance against current single drugs.

"Therapy for many types of cancers are expected to increasingly rely upon rational combinations of

agents," said Dr. Mark Fishman, President of the Novartis Institutes for BioMedical Research. "Immunotherapy agents provide additional arrows in our quiver for such combinations. They complement our extensive portfolio of drugs that hit genetically-defined cancer-causing pathways, and also may be relevant to expansion of CAR therapies." Financial terms are not disclosed. (Source: Novartis Website, 17 February, 2014)

Regulatory and Legislative Highlights

by Deepen Joshi, Sunovion Pharmaceuticals

Regulatory and Legislative Highlights, is a regular feature of the Boston Area Chapter Newsletter. It reviews recent actions by the FDA and other regulatory agencies and governmental bodies, both federal and regional, with the potential to impact the pharma, biotech and device industries, and related fields.

FDA Approved 27 Novel New Drugs in 2013

In 2013, FDA's Center for Drug Evaluation and Research (CDER) approved 27 novel new medicines, known as new molecular entities (NMEs) under both New Drug Applications (NDAs) and Biologics License Applications (BLAs). NMEs are often innovative new products that serve previously unmet medical needs or otherwise significantly help to advance patient care and public health. However, in some cases, while categorized as novel for technical and/or administrative purposes, a particular NME may not necessarily offer unique clinical advantages over existing therapies.

CDER approved 27 NMEs in 2013, which is similar to average totals of other years from this time period. For instance from 2004 through 2012, CDER has averaged about 26 NME approvals per year. In 2012, CDER approved 39 NMEs, but this was an unusually high number compared to any other total in more than a decade.

The number of applications CDER has been receiving for NMEs has not been consistently and significantly increasing. From 2004 through 2012, CDER filed an average of about 34 applications for NMEs per year. CDER projects about 36 for 2013, which is consistent with previous years in this decade. With a relatively steady number of applications coming in over time, it is noteworthy that FDA cannot expect a continuing upward trend for NME approvals until a sustained increase in the number of applications for NMEs submitted for approval is also demonstrated. (Source: 2013 Novel New Drugs Summary, FDA Center for Drug Evaluation and Research, January 2014)

FDA Approves Vimizim to Treat Rare Congenital Enzyme Disorder

The FDA approved Vimizim (elosulfase alfa), the first FDA-approved treatment for Mucopolysaccharidosis Type IVA (Morquio A syndrome). Morquio A syndrome is a rare, autosomal recessive lysosomal storage disease caused by a deficiency in N-acetylgalactosamine-6-sulfate sulfatase (GALNS). Vimizim is intended to replace the missing GALNS enzyme involved in an important metabolic pathway. Absence of this enzyme leads to problems with bone development, growth and mobility. There are approximately 800 patients with Morquio A syndrome in the United States.

Vimizim was granted priority review. An FDA priority review provides for an expedited review of drugs for serious diseases or conditions that may offer major advances in treatment. Vimizim is also the first drug to receive the Rare Pediatric Disease Priority Review Voucher - a provision that aims to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. Vimizim is marketed by Novato, California-based BioMarin Pharmaceutical Inc. (Source: FDA Website, 14 February, 2014)

FDA Initiates Secure Supply Chain Pilot Program to Enhance Security of Imported Drugs

The FDA announced the initiation of the Secure Supply Chain Pilot Program to enhance the security of imported drugs. In August 2013, the FDA published a notice in the Federal Register (78 FR 51192) to solicit companies to voluntarily submit applications for participation in this two-year program. Thirteen prequalified companies have now been designated to take part, and will receive expedited entry for the importation of up to five selected drug products into the United States.

The goal of the program is to enable the FDA to evaluate resource savings that will allow the agency to focus imports surveillance resources on preventing the entry of high-risk drugs that are the most likely to compromise the quality and safety of the U.S. drug supply. (Source: FDA Website, 18 February, 2014)

FDA and European Medicines Agency Strengthen Collaboration in Medicine Safety

The FDA and the European Medicines Agency (EMA) have set-up a new 'cluster' on pharmacovigilance (medicine safety) topics. Clusters are regular collaborative meetings between the EMA and regulators outside of the European Union which focus on specific topic areas that have been identified as requiring an intensified exchange of information and collaboration. Building on the experience of previous regular videoconferences between the FDA and the EMA in this area and on the recent creation of the EMA's Pharmacovigilance Risk Assessment Committee, this cluster will provide a forum for a more systematic and focused exchange of information on the safety of medicines.

The FDA and the EMA have already set-up such clusters to discuss issues related to biosimilars,

medicines to treat cancer, orpnan medicines, medicines for children and blood-based products, among other topics. Health Canada and the Japanese Pharmaceuticals and Medical Devices Agency are also involved in some of these clusters.

As part of the new cluster, discussions on shared pharmacovigilance issues will now take place between the agencies on a monthly basis by teleconference. This increased degree of interaction will allow the agencies to work swiftly in the area of the safety of medicines and to coordinate communication activities. The creation of this cluster is the latest step in the FDA's and the EMA's broader approach to expand and reinforce international collaboration. (Source: FDA Website, 19 February, 2014)

FDA Approves Myalept to Treat Rare Metabolic Disease

The FDA approved Myalept (metreleptin for injection) as replacement therapy to treat the complications of leptin deficiency, in addition to diet, in patients with congenital generalized or acquired generalized lipodystrophy. Myalept is marketed by San Diego-based Amylin Pharmaceuticals.

Generalized lipodystrophy is a condition associated with a lack of fat tissue. Patients with congenital generalized lipodystrophy are born with little or no fat tissue. Patients with acquired generalized lipodystrophy generally lose fat tissue over time. Because the hormone leptin is made by fat tissue, patients with generalized lipodystrophy have very low leptin levels. Leptin regulates food intake and other hormones, such as insulin. Patients with both types of generalized lipodystrophy often develop severe insulin resistance at a young age and may have diabetes mellitus that is difficult to control or very high levels of triglycerides in the blood (hypertriglyceridemia) that can lead to inflammation of the pancreas. (Source: FDA Website, 25 February, 2014)

FDA Approves Otezla to Treat Psoriatic Arthritis

The FDA approved Otezla (apremilast) to treat adults with active psoriatic arthritis (PsA). Otezla is manufactured for Celgene Corporation, Summit, N.J.

PsA is a form of arthritis that affects some people with psoriasis. Most people develop psoriasis first and are later diagnosed with PsA. Joint pain, stiffness and swelling are the main signs and symptoms of PsA. Currently approved treatments for PsA include corticosteroids, tumor necrosis factor (TNF) blockers, and an interleukin-12/interleukin-23 inhibitor.

Patients treated with Otezla should have their weight monitored regularly by a healthcare professional. If unexplained or clinically significant weight loss occurs, the weight loss should be evaluated and discontinuation of treatment should be considered. Treatment with Otezla was also associated with an increase in reports of depression compared to placebo.

The FDA is requiring a pregnancy exposure registry as a post-marketing requirement to assess the risks to pregnant women related to Otezla exposure. (Source: FDA Website, 21 March, 2014)

FDA Approves Topamax for Migraine Prevention in Adolescents

The FDA approved Topamax (topiramate) for prevention (prophylaxis) of migraine headaches in adolescents ages 12 to 17. This is the first FDA approval of a drug for migraine prevention in this age group. The medication is taken on a daily basis to reduce the frequency of migraine headaches. Topamax was first approved by the FDA in 1996 to prevent seizures. It was approved for migraine prevention in adults in 2004. Topamax is manufactured by Janssen Pharmaceuticals, Inc. of Titusville, N.J.

About 12 percent of the U.S. population experiences migraine headaches. Migraine headaches are characterized by episodes of throbbing and pulsating pain in the head, and may occur several times per month. Other common symptoms include increased sensitivity to light, noise, and odors, as well as nausea and vomiting. Many patients experience their first migraine attack before reaching adulthood, and migraine can be just as disabling in teens as it is in adults.

Topamax increases the risk of the development of cleft lip and/or cleft palate (oral clefts) in infants born to women who take the drug during pregnancy. The benefits and risks of Topamax should be carefully weighed before using it in women of childbearing age. If the decision is made to use the medication by a woman of childbearing age, effective birth control should be used. (Source: FDA Website, 28 March, 2014)

Biogen Idec Receives FDA Approval for Hemophilia B Treatment

The FDA approved Alprolix, Coagulation Factor IX (Recombinant), Fc Fusion Protein, for use in adults and children who have Hemophilia B. Alprolix is the first Hemophilia B treatment designed to require less frequent injections when used to prevent or reduce the frequency of bleeding. Alprolix is manufactured by Biogen Idec and received orphan-drug designation for this use because it is intended for treatment of a rare disease or condition. Alprolix is approved to help control and prevent bleeding episodes, manage bleeding during surgical procedures, and prevent or reduce the frequency of bleeding episodes (prophylaxis). Alprolix consists of the Factor IX molecule linked to a protein fragment, Fc, which is found in antibodies. This makes the product last longer in circulation.

Hemophilia B is an inherited sex-linked, blood-clotting disorder, which primarily affects males, and is caused by defects in the Factor IX gene. Hemophilia B affects about 3,300 people in the United States. People with Hemophilia B can experience repeated episodes of potentially serious bleeding, mainly

into the joints, which can be destroyed by the bleeding. (Source: FDA Website, 28 March, 2014)

FDA Approves GSK's Tanzeum to Treat Type 2 Diabetes

The FDA approved Tanzeum (albiglutide) subcutaneous injection to improve glycemic control, along with diet and exercise, in adults with type 2 diabetes. Type 2 diabetes affects approximately 24 million people and accounts for more than 90 percent of diabetes cases diagnosed in the United States. Over time, high blood sugar levels can increase the risk for serious complications, including heart disease, blindness, and nerve and kidney damage. Tanzeum is manufactured by GlaxoSmithKline of Wilmington, Delaware.

Tanzeum is a glucagon-like peptide-1 (GLP-1) receptor agonist, a hormone that helps normalize blood sugar levels. The drug's safety and effectiveness were evaluated in eight clinical trials involving more than 2,000 patients with type 2 diabetes. Patients participating in the trials showed an improvement in their HbA1c level (hemoglobin A1c or glycosylated hemoglobin, a measure of blood sugar control).

Tanzeum has a Boxed Warning to warn that tumors of the thyroid gland (thyroid C-cell tumors) have been observed in rodent studies with some GLP-1 receptor agonists, but that it is unknown whether Tanzeum causes thyroid C-cell tumors, including a type of thyroid cancer called medullary thyroid carcinoma (MTC), in humans. Tanzeum should not be used in patients with a personal or family history of MTC or in patients with Multiple Endocrine Neoplasia syndrome type 2 (a disease where patients have tumors in more than one gland in their body and that predisposes them to MTC).

The FDA is requiring the following post-marketing studies for Tanzeum:

- clinical trial to evaluate dosing, efficacy, and safety in pediatric patients;
- medullary thyroid carcinoma (MTC) case registry of at least 15 years duration to identify any increase in MTC incidence related to Tanzeum;
- cardiovascular outcomes trial (CVOT) to evaluate the cardiovascular risk of Tanzeum in patients with high baseline risk of cardiovascular disease.

The FDA approved Tanzeum with a Risk Evaluation and Mitigation Strategy (REMS), which consists of a communication plan to inform health care providers about the serious risks associated with Tanzeum. (Source: FDA Website, 15 April, 2014)

New Members

Mrs. Sandra J. Ahern, MSc, MBA, Director Clinical Supply Quality, Genzyme

Joy A. Ajayi-Carrier, Graduate Student, Tufts University

Vicheth Ang, Sr., C&Q Engineer, DPS Engineering

Plamen Atanassov, Student, University of Massachusetts Lowell

Mr. Benjamin J. Baranowski, Associate Engineer, Mylan Technologies Inc

Mr. Cole M. Belanger, Student, University of Massachusetts Dartmouth

Ryan Bogosian, Student, University of New Hampshire

Mr. Kevin J. Bowen, Senior Quality Assurance Specialist, ARIAD Pharmaceuticals

Timothy J. Buonodono, Student, UMass Lowell

Lora M. Cameron-Landis, Senior Consultant, Hyde Engineering + Consulting

Mr. Matthew Cataldo, Dir. Business Development, JLL Constructions

Mr. Prasun Chatterjee, Student, University of Massachusetts Dartmouth

Bryan YJ Choi, Graduate Researcher, Tufts University

Ms. Jane E. Chuprin, Student, Boston University

Mr. Paul Collins, Manager Drug Manufacturing, DUSA Pharmaceuticals

Mr. Damian B. Costa, Student, Umass Dartmouth

Eben Crawford

Ms. Sherine Farida Dao, University of Massachusetts Lowell

Willow DiLuzio, Millennium

Mr. Francis Dumont, Sr. Director, Pfizer Inc

Joseph M. Dunlavey, Student, Massachusetts Maritime Academy

Dr. Qinguo Fan, Professor, University of Massachusetts Dartmouth

Ms. Laura R. Forrest, Albany College of Pharmacy and Health Sciences

Ms. Jana E. Frenier, Information Research Specialist, PAREXEL International

Ms. Emily Geishecker, Research Assistant, Lehigh University

Mr. Ronn Gerra, Capital Project Manager, Shire HGT

Adam Giandomenico, President, Particles Plus, Inc.

Amy D. Giles, Quality Specialist, Rhodes Pharmaceuticals

Ms. Nafsika Gjika, Student, Northeastern University

Mr. Joshua D. Grims, Lead Engineer, Mylan Technologies Inc

Vincent Guido, Customer Service, Terracon Corporation

Mr. Christopher B. Hack, President, King Gage Engineering

Mr. Omar Janoudi, Student, Boston University

Mr. Derek A. Jones, Student, Northeastern University

Seth Joshua Kamens, Student, Worcester Polytechnic Institute

Mr. Jeffrey John Kane, Lecturer, University of Massachusetts

Mr. Antoine G. Khoury, MCPHS University

Mr. Christopher M. Kilday, Facilities Engineering Manager, Whitehead Institute

Mr. Adam J. Laber, Consultant, ICQ Consultants

Mr. Tyler J.M. Latour, Student, University of Massachusetts Dartmouth

Ms. Rachel Le, University of Massachusetts Dartmouth

Mr. Eric W. Loreaux, Boston University

Brian Loynd, CRB Builders, LLC

Mr. Patrick L. McCarthy, Student, University of Massachusetts Dartmouth

Mr. Matthew J. McNulty, Associate Process Engineer, Genzyme

Erwin Melgar, Senior Pharmaceutical Engineer, Hyde Engineering Consulting Dr. Kathleen Mihlbachler, Global Director of Process Separation, LEWA Process Technology

Adam C. Miller, Student, Northeastern University

Mr. Cody J. - R. Montelo, United States Soldier/Full Time Student, University of Massachusetts Darmouth

Mr. Rakesh Motani, Process Engineer III, Genzyme Corporation

Rajutsha Nath, Student, Massachusetts College of Pharmacy and Health Sciences University

Dr. Mary Nauffal, Student, Northeastern University

Mr. Vasker Neupane, Student, University of Massachusetts Dartmouth

Brendan Nichols, Student, University of New Hampshire

Hugh O'Connor, Senior Plant Engineer, Shire HGT

Mrs. Cherie A. Parkhurst-Lang, Sr Assoc Scientist, Biogen Idec

Robert Parody, Associate Professor, Rochester Institute of Technology

Mr. Shrinesh Nitesh Patel, Boston University

Nicholas A. Phillips, University of Massachusetts Dartmouth

Brian A. Pink, Student, Tufts

Mr. John Putnam, Advanced Controls Specialist, Consultant

Husein J. Rokadia, Validation Engineer, Commissioning Agents Inc.

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Mr. Ryan P. A. Sullivan, Student, Nipmuc Regional High School

Linlin Sun, PhD Student, Northeastern University

Mr. Grea Tedeschi. Marketina. vacuubrand

Hieu M. Tran, Student, Boston University

Ngan Tran, Student, Northeastern University

Mr. Joshua Trowbridge, Student, Northeastern University

Haleh Valian, BiogenIdec

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Mr. Gerard W. Walsh, Jr., Sr. Project Manager, Shire

Mr. Mark W. Watson, Senior Director, Manufacturing Technology & Development, Lantheus Medical Imaging

Ms. Meghan A. Wiebe, Process Improvement Engineer, Eastman Chemical

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- Dr. Donald L. Towns, Donald L Towns Professional Engineer
- Mr. Lawrence W. Weiner, Biogen Idec
- Mr. Michael H. Welch, Pneumatic Scale Angelus
- Mr. Jack N. Wentz, Lantheus
- Mr. Jeffrey L. Werner, BFS Pharma Inc
- Mr. James L. Whalen, Watson-Marlow Flexicon
- Mr. David A. Wilson, Abbott Bioresearch Center
- Mr. Jay F. Zaino, GxP Automation LLC
- Mr. Gary V. Zoccolante, Siemens Water Technologies Corp

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<u>10 Year Anniversary</u>

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Mr. Mark Bryan, GXP Automation LLC
Mr. Brian Chviruk, P.E., Shire
Mr. Steve J. Comeau, Hallam-ICS
Dr. Michael Henson, University of Massachusetts Amherst
Ms. Carolyn B. Morgan, Astro Pak Corp
Ms. Susan M. Murphy, SNC-Lavalin
Mr. Lonn S. Rider, Rhodes Technologies

5 Year Anniversary

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Ms. Doreen Newhouse,

Mr. David J. Stack, Parker Hannifin

Mr. Paul D. Sullivan, RW Sullivan Engineering

Wasim Syed, Shire HGT

Mr. Wentao Wang, Pfizer

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