

Psoriasis Drug Approval

In September the FDA approved Stelara ustekinumab, a monoclonal antibody (MAb) for adults with moderate to severe psoriasis. This biologic treats psoriasis by blocking the action of two proteins that contribute to the overproduction of skin cells and inflammation.

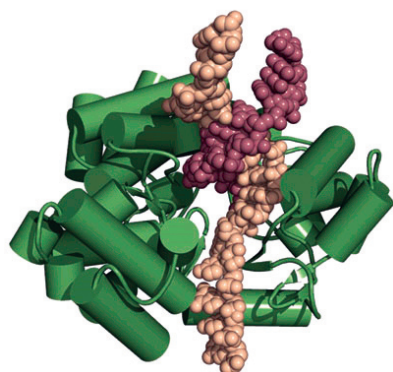
The approval came with strings attached, however. The drug maker Centocor Ortho Biotech Inc. (a subsidiary of Johnson & Johnson) will be required to complete a risk evaluation and mitigation strategy (REMS) that includes a communication plan targeted to healthcare providers as well as a medication guide for patients.

Studies of Telomeres Win Nobel Prize

The Royal Swedish Academy of Sciences announced on 5 October that the 2009 Nobel Prize in Physiology or Medicine was awarded to Jack W. Szostak (a Howard Hughes Medical Institute investigator at Massachusetts General Hospital and Harvard Medical School), Elizabeth Blackburn (of the University of California, San Francisco), and Carol Greider (of the Johns Hopkins University School of Medicine). These three scientists were honored "for the discovery of how chromosomes are protected by telomeres and the enzyme telomerase," according to a Howard Hughes Medical Institute release.

The three honorees solved a major puzzle in biology: how chromosomes can be copied exactly and completely during

Telomerase model solved by Emmanuel Skordalakes, a researcher at The Wistar Institute. WWW.WISTAR.ORG



cell divisions and how they are protected against degradation. The scientists are being recognized for their discovery of the specialized process by which the ends of chromosomes are synthesized and for their discovery of the enzyme telomerase. This work revealed how organisms rely on the enzyme to protect their genome from degradation, and it laid the groundwork for later studies linking telomerase to cancer and aging-related ailments in humans.

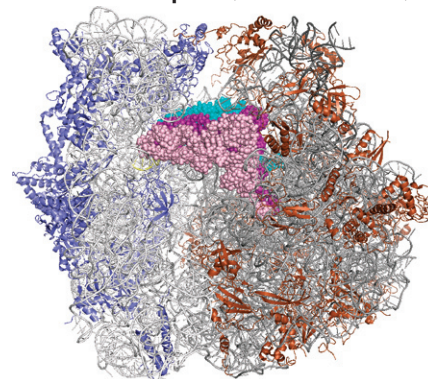
Blackburn and Szostak discovered that it is a unique DNA sequence in the telomeres that protects chromosomes from degradation. Blackburn and Greider identified telomerase, the enzyme that makes telomere DNA. To prevent all cells from becoming cancerous, adult cells are equipped with a record keeping system that lets them know how many times they have multiplied. At some preset limit, often around 80 divisions, the cell dies. Telomerase interferes with that record keeping. Drugs that interfere with telomerase might be able to control the growth of cancer cells. Such drugs are only now being explored and tested, nearly 20 years after the Nobel recipients' discovery.

According to a release from EuropaBio, "These discoveries have had a major impact within the scientific community at large because, for some time, scientists have speculated that telomere shortening could be the reason for aging of cells and organisms. Because most normal cells do not divide frequently, their chromosomes are not at risk of shortening and they do not require high telomerase activity. However, in contrast, cancer cells have the ability to divide infinitely and yet preserve their telomeres. Further studies can now be undertaken to establish the significance of this gene sequence and enzyme in the replication of malignant and healthy cells. Several studies are already under way in this area, including clinical trials evaluating vaccines directed against cells with elevated telomerase activity."

Chemistry Nobel Prize for Study of Ribosomes

The Royal Swedish Academy of Sciences on 7 October named Thomas A. Steitz (a Howard Hughes Medical Institute

Ribosome model screen capture from the online Proteopedia (WWW.PROTEOPEDIA.ORG)



investigator at Yale University), Venkatraman Ramakrishnan (of the Medical Research Council Laboratory of Molecular Biology), and Ada E. Yonath (of the Weizmann Institute of Science) as recipients of the 2009 Nobel Prize in Chemistry for their studies of the structure and function of the ribosome.

The prize was awarded to the three scientists for research that shows what the ribosome looks like and how it functions at the atomic level. All three used X-ray crystallography to map the position for each and every one of the hundreds of thousands of atoms that make up the ribosome. For the full story and additional background information, please go to <http://www.hhmi.org/news/steitz20091007.html>.

BPSA Goes Solo

The Bio-Process Systems Alliance (BPSA) announced on 6 October that it will realign its growing industry trade group with the pharmaceutical and biotechnology business communities. The group was originally formed within the plastics industry in 2005 as a business unit of SPI, The Plastics Industry Trade Association. Now it reestablishes itself as an independent trade association and continues its mission to encourage and accelerate the adoption and implementation of single-use manufacturing systems in the biopharmaceuticals industry.

"It was a difficult decision to move outside the traditional plastics industry," said BPSA Chairman Jerry Martin, senior vice president of scientific affairs at Pall

Life Sciences. "As highly specialized plastics manufacturers, our charter member companies found SPI to be an excellent host organization for the guidance and administrative services we required for our launch and infancy. Our needs began to shift in 2008, however, when BPSA started accepting biopharmaceutical and biotech companies into our membership ranks to establish a safe-harbor information exchange between end users of single-use systems and suppliers. Single-use technology end user companies fall outside of SPI's core constituency."

"The timing is ideal for both organizations," added Ken Bibbo, BPSA treasurer and vice president of operations for HyNetics Corporation. "At this stage in our growth cycle, BPSA is ready to seize new opportunities in the biopharmaceutical world just as SPI is honing its core missions of advocacy, communications, and business development initiatives for plastics. It is an exhilarating time of change — I know my company is excited to remain a member of both groups."

"The BPSA is at the cutting edge of a sector poised for growth in the bioprocessing industry," said SPI President and CEO Bill Carteau. "SPI is proud to have served as the group's birthplace, and it was a pleasure to provide our legal, regulatory, and marketing expertise during its critical formative years. We are eager to maintain an affiliation and will seek out ways we can collaborate on joint programs that will bring mutual benefit to both organizations."

Rare Disease Organizations Form Strategic Alliance

Two major advocacy groups for people with rare diseases — the National Organization for Rare Disorders (NORD, www.rare diseases.org) in the United States and the European Organization for Rare Diseases (EURORDIS, www.eurordis.org) — have joined forces to work on behalf of rare disease patients and their families in both regions. These groups are nongovernmental (NGO), patient-driven alliances of patient organizations and individuals dedicated to improving the lives of people with rare diseases.

This collaboration follows an announcement by the US FDA and the EMEA of a collaborative effort to ensure appropriate conduct of clinical trials and adoption of a common application for orphan drug products — those being

US FDA Issues REMS Draft Guidance

On 30 September, the FDA issued its first draft guidance on risk evaluation and mitigation strategies for pharmaceutical products linked to serious adverse events. Dow Jones Newswires reported that companies can be fined up to \$10 million for noncompliance.

Titled *Format and Content of Proposed Risk Evaluation and Mitigation Strategies (REMS), REMS Assessments, and Proposed REMS Modifications*, the guidance document can be found online at www.fda.gov/downloads/Drugs/complianceRegulatoryInformation/Guidances/UCM184128.pdf.

An FDA news release highlights its main points:

- provides the agency's current thinking on the format and content that

industry should use for submissions of proposed REMS

- describes each potential element
- includes preliminary information on the content of assessments and proposed modifications of approved REMS

- describes REMS policies for certain regulatory situations

- informs industry about who to contact within FDA about a REMS

- indicates FDA websites where documents about approved REMS will be posted

- provides an example of what an approved REMS might look like for a fictitious product.

Future draft guidances will address additional REMS topics.

developed to treat rare diseases. The intent is to increase global awareness, promote research and the development of new treatments, and provide advocacy for more compassionate public policies, according to press releases from the NGOs.

A disease is considered rare in the United States if it affects <200,000 Americans. Almost 30 million (nearly one in 10) Americans have a rare disease. In the European Union, a disease is considered rare if it affects fewer than one in 2,000 people. There are 30 million Europeans with rare diseases.

Roche Rebrands

Roche announced in October that its brand in the United States will be replaced by the Genentech brand on all products ranging from traditional small-molecule drugs to biotech treatments. Updated business cards for the US sales force feature the Genentech logo in place of Roche's with the phrase, "A member of the Roche Group" included.

Forecasting Success

Pharmer's Market — a recently launched collaboration among MIT, Harvard, and Crowdcast prediction markets (www.crowdcast.com) — is the first prediction market for pharmaceuticals. The website (<https://pharmersmarket.crowdcast.com>) harnesses collective intelligence to predict the likelihood of drugs succeeding through the three phases of clinical trials. This "prediction market" launches by examining six drugs that are

currently in clinical trials for the treatment of breast cancer. It is designed to use the intelligence of the pharmaceutical and medical community at large to predict the success or failure of drugs progressing through the phases of testing.

In addition to helping predict the outcome of clinical trials, the Pharmer's Market will also help doctors and patients gain insights into the safety and efficacy of specific drugs at the early stages of clinical trials beyond information that is publicly available. The website is now live and inviting participation.

Outsourcing Award Repeat

Catalent Pharma Solutions won for the second year in a row the "Best Contract Manufacturing Project" award at this year's European Outsourcing Awards ceremony. The company was recognized for supporting GlaxoSmithKline's efforts to meet global demand for a key antiviral drug recommended to treat H1N1 influenza. Launched in 2005, the annual award ceremony recognizes significant developments in contract services and rewards successful companies for their outstanding contributions and achievements throughout the year. Other 2009 award recipients include SAFC, Baxter, and Solvias AG. Full award results can be found online at www.europeanoutsourcingawards.com/outourcing/winners09.html. Entries for the 2010 awards must be received by 1 July 2010, and more information is available on the website. 🌐