News From the Food and Drug Administration

Notable Year for Novel Drugs
During 2017, the FDA’s Center for Drug Evaluation and Research (CDER) ushered 46 novel drugs through the approval process, the highest number per year over the last 10 years.

Since 2008, CDER has averaged about 31 novel drug approvals per year. The low during that time period was in 2010, when 21 novel drugs were approved. About one-third of the novel drugs approved last year were first in their class medications, which often have different mechanisms of action than existing therapies. Those approved last year included dupilumab for adults with moderate to severe eczema and ocrelizumab for adults with relapsing forms of multiple sclerosis (MS) and primary progressive MS.

Nearly 40% of last year’s novel drugs were approved for rare, so-called orphan diseases that affect 200,000 or fewer US residents. Among them were cerliponase alfa for a specific form of Batten disease, which can cause severe neurological impairments, and emicizumab, the first nonblood product approved to prevent or reduce bleeding in certain patients with hemophilia A.

In addition to novel drugs, last year’s FDA drug approvals included 5 biosimilars, which have no clinically meaningful differences in safety and effectiveness compared with their already approved counterparts. The agency also approved 3 abuse-deterrent formulations of opioid pain medications already on the market—oxycodeone, hydrocodone, and morphine sulfate.

Diabetic Foot Ulcers Heal With Shock Wave Therapy
The FDA has approved the first shock wave device indicated for patients with diabetic foot ulcers.

Marketed as the Dermapace System, the device uses a handheld probe to deliver high-energy pulses similar to sound waves to the wound’s surface. According to the manufacturer, Sanuwave Health Inc, of Alpharetta, Georgia, the device increases perfusion and arteriogenesis, angiogenesis, biofilm disruption, and growth factor upregulation, which help regenerate skin and musculoskeletal and vascular structures.

Treatment consists of 4 to 8 brief, non-invasive applications given over 2 to 10 weeks. Monitoring and usual care are required afterward. The device is indicated for adults with diabetes who have foot ulcers lasting more than 30 days. The FDA noted that about 25% of people with diabetes will develop a foot ulcer. Poor circulation or a foot infection that doesn’t respond to treatment sometimes results in amputation.

Officials at the FDA said the agency reviewed clinical data from 2 trials that involved 336 patients with diabetes. The treatment group received usual care consisting of wet-to-dry dressings or debridement and the shock wave therapy. The control group was treated with usual care and sham shock wave therapy. Some patients’ blood glucose was well controlled while others’ blood glucose was not.

The trials’ primary end point was complete wound closure—full skin reepithelialization without the need for drainage or dressings—within 12 weeks of starting treatment. After 24 weeks, 44% of patients in the Dermapace group who received 1 to 7 treatments met the end point compared with 30% in the control group.

The most common adverse events associated with the Dermapace System were pain during treatment, local bruising and numbness, migraine headaches, nausea, syncope, wound infection, cellulitis, osteomyelitis, and fever.

Crackdown on Stem Cell Product
A New Jersey-based company that processes and stores stem cell products derived from human adipose tissue has run aground of FDA regulations.

In a warning letter sent last month, the agency notified the American CryoStem Corporation of Eatontown, New Jersey, that its product, Atcell, doesn’t meet criteria that exempt some human cellular and tissue-based products from the FDA approval process. To bypass approval, structural tissues like human fat must meet “minimal manipulation” criteria: they cannot be processed in a way that changes their original form beyond what could safely be used in reconstruction, repair, or replacement procedures.

During an inspection of CryoStem’s facilities in July 2017, FDA officials said they documented how the company processes adipose tissue into stromal vascular fraction (SVF), a mix of cell populations that remain after blood and fat components are removed. The manufacturing process then expands SVF through cell culture to produce Atcell. Even though Atcell is intended for autologous use, the FDA said the manufacturing process didn’t meet minimal manipulation criteria and contamination could have occurred. The agency’s warning letter also pointed out that Atcell is not properly licensed as a biologic.

In November 2017, the FDA announced new plans to oversee regenerative medicine products, including cellular therapies. “We’re going to be stepping up enforcement activities against those who manufacture and market products in ways that put patients risk,” FDA Commissioner Scott Gottlieb, MD, said in a statement.

John Arnone, American CryoStem’s chief executive officer, said in a statement that the company takes the FDA’s warning very seriously and would meet the FDA’s deadline for a response. “We will actively engage with the agency to resolve these issues and continue to improve our quality systems and processes as an organization-wide priority,” Arnone said. – Rebecca Voelker, MSJ

Note: Source references are available online through hyperlinks embedded in the article text.