Orphan Drug Updates

Recent Drug Approvals

- Anthim (obiltoxaximab) injection has been approved to treat inhalational anthrax in combination with appropriate antibacterial drugs and to prevent inhalational anthrax when alternative therapies are not available or not appropriate. Anthim was developed by Elusys Therapeutics, Inc. in conjunction with the U.S. Department of Health and Human Services’ Biomedical Advanced Research and Development Authority. Read the press release.
- Kovaltry antihemophilic factor (recombinant), an unmodified, full-length factor VIII compound, has been approved for the treatment of hemophilia A in children and adults. Kovaltry is manufactured by Bayer. More.
- Idelvion, coagulation Factor IX (recombinant), albumin fusion protein, has been approved for use in children and adults with hemophilia B. Idelvion is manufactured by CSL Behring. Read the press release.
- Xalkori (crizotinib) has been approved to treat people with advanced non-small cell lung cancer (NSCLC) whose tumors have an ROS-1 gene alteration. Xalkori is the first and only FDA approved treatment for patients with ROS-1 positive NSCLC. Xalkori is marketed by Pfizer. Read the press release.
- Everolimus (Afinitor) has been approved for the treatment of adult patients with progressive, well-differentiated non-functional, neuroendocrine tumors of gastrointestinal or lung origin with unresectable, locally advanced or metastatic disease. Afinitor is manufactured by Novartis. More.
- Obinutuzumab (Gazyva) has been approved for use in combination with bendamustine followed by obinutuzumab monotherapy for the treatment of patients with follicular lymphoma who relapsed after, or are refractory to, a rituximab-containing regimen. Obinutuzumab was previously approved for use in combination with chlorambucil for the treatment of patients with previously untreated chronic lymphocytic leukemia and is manufactured by Genentech. More.

Recent Orphan Drug Designations

- DelMar Pharmaceuticals’ VAL-083 (dianhydrogalactitol) for the treatment of medulloblastoma
- Soricimed Biopharma for its peptide SOR-C13 for treatment of ovarian cancer
- ProNAi Therapeutics’ PNT2258 for the treatment of diffuse large B-cell lymphoma
- MedImmune for MEDI-551 to treat neuromyelitis optica
- Sellas Life Sciences’ WT1 cancer vaccine for the treatment of malignant pleural mesothelioma
- BioMarin Pharmaceutical Inc. for a gene therapy intended for hemophilia A

Prepared by National Organization for Rare Disorders® (NORD®). For more information, contact education@rarediseases.org
Recent Humanitarian Use Designation

- Xeltis for its bioabsorbable cardiac pulmonary valve to treat children with congenital obstruction by correcting or reconstructing the right ventricular outflow tract

Recent Fast Track Designations

- Nivalis Therapeutics for its N91115, a stabilizer of the cystic fibrosis transmembrane conductance regulator protein
- Omeros’ OMS721 for the treatment of atypical hemolytic uremic syndrome