ANSEA
YOUR ACCESS PARTNER

ANSEA INSIGHTS

Newsletter 2025 | January Edition

Dear Valued Client,

Happy New Year! We're thrilled to introduce our first edition of ANSEA Insights, the latest initiative to stay connected and share valuable insights with you. We extend our warmest wishes for a successful and prosperous 2025. Here's to embracing new opportunities and fostering continued collaboration.

At ANSEA, we recognize the importance of staying ahead of the curve in the rapidly evolving healthcare landscape. As part of our commitment to support your business goals, we have provided a collage of key developments and innovations shaping the global healthcare industry.

With our expertise expanding into diverse markets, we have developed a collection of insights to address emerging opportunities. This newsletter highlights important updates across four key themes: a) Healthcare Trends to Watch; b) Pharma M&A Activity: Recent Deals and Insights; and c) Regulatory Updates d) Recent Drug Approvals: Advancing Patient Care

We hope these insights provide valuable perspectives as you navigate the ever-changing healthcare environment.

Healthcare Trends to Watch

Recent healthcare trends underscore the transformative potential of biologics in addressing complex diseases through personalized and targeted therapies. Alongside advancements in precision medicine and the rising demand for effective weight loss treatments, biologics are driving innovation in chronic disease management, genetic disorders, and obesity-related conditions.

1. Precision Medicine

Precision medicine is transforming healthcare by tailoring treatments to individual patients based on their genetic, environmental, and lifestyle factors. This approach enables more targeted therapies, improving outcomes and reducing side effects.

Since 2023, AstraZeneca has applied precision medicine to 90% of its R&D portfolio, focusing on oncology and chronic diseases. By using biomarkers, they identify patients most likely to benefit from specific treatments, improving clinical trial success rates and therapy precision. [1]

In 2023, the FDA approved Casgevy, the first CRISPR-Cas9-based therapy for sickle cell disease. Developed by Vertex Pharmaceuticals and CRISPR Therapeutics, this one-time treatment targets the genetic mutations causing the disease, offering a potentially curative solution for patients with recurrent vaso-occlusive crises. This milestone highlights the power of gene editing in addressing complex genetic disorders. [2]

2. Rise of Weight Loss Medications

The demand for weight loss medications is surging due to rising obesity rates, increased awareness of health risks, and the need for effective treatments.

Eli Lilly's Zepbound® (Tirzepatide): A dual GIP and GLP-1 receptor agonist, Zepbound® delivered significant results in December 2024 clinical trials, achieving 20.2% average weight loss over 72 weeks (50.3 lbs/22.8 kg), outperforming Wegovy®. [3]

Amgen's MariTide (November 2024): With a less frequent dosing schedule, MariTide showed up to 20% weight loss in Phase 2 studies without plateauing. It also improved cardiometabolic markers, including HbA1c reductions in patients with type 2 diabetes and obesity, highlighting its potential as a comprehensive treatment. [4]

3. **Increasing Adoption of Biologics**

   Biologics, including monoclonal antibodies, vaccines, and gene therapies, are becoming significant in modern medicine for treating complex and chronic diseases. These therapies, derived from living organisms, offer targeted and effective solutions, driven by advancements in biotechnology and supportive regulations.

   Ozempic (Semaglutide): A monoclonal antibody for type 2 diabetes management and weight loss, highlighting biologics' role in chronic disease care. [5]

   Zolgensma (Onasemnogene abeparvovec): A gene therapy for spinal muscular atrophy, addressing the disease's genetic cause and marking a breakthrough in treating genetic disorders. [6]

   Partners can capitalize on precision medicine, weight loss treatments, and biologics by leveraging biomarker-driven approaches, gene-editing technologies, and advanced biotechnology. Innovations like Casgevy, Zepbound®, MariTide, Ozempic, and Zolgensma highlight opportunities to address chronic, genetic, and obesity-related diseases, emphasizing collaboration's role in driving innovation and improving patient outcomes.

Pharma M&A Activity: Recent Deals and Insights

The recent pharma M&A wave highlights a shift toward specialized therapies, strategic partnerships, and targeted investments, as seen in Novo Nordisk's acquisition of Catalent and BMS's purchase of Karuna Therapeutics. This reflects the industry's focus on enhancing capabilities and pipelines to meet evolving market dynamics and patient needs.

1. **Novo Nordisk's $16.5 Billion Acquisition of Catalent**

   In one of 2024's largest acquisitions, Novo Holdings plans to acquire CDMO Catalent for $16.5 billion to expand manufacturing capacity for its GLP-1 agonists for Type 2 diabetes and obesity. This move highlights the increasing importance of CDMOs in the pharma industry, allowing Novo Nordisk to secure its supply chain and ensure a steady drug supply.[7]

2. **Bristol-Myers Squibb's $14 Billion Acquisition of Karuna Therapeutics**

   Bristol-Myers Squibb acquired Karuna Therapeutics for $14 billion, enhancing its portfolio with KarXT, an antipsychotic drug for schizophrenia under FDA review. This acquisition highlights growing interest in CNS therapies, expanding BMS's presence in the field and offering a potential blockbuster drug.[8]

3. **Astellas and AviadoBio Partnership**

   Astellas Pharma partnered with AviadoBio to develop a gene therapy for a neurodegenerative disease, with AviadoBio receiving $30 million upfront and up to $2.18 billion in milestone payments. This collaboration underscores the growing focus on gene therapies for neurodegenerative diseases and increasing pharma-biotech partnerships.[9]

   Recent pharma acquisitions and partnerships highlight key opportunities for expansion and competitiveness. Novo Nordisk's $16.5 billion acquisition of Catalent boosts GLP-1 agonist manufacturing, while BMS's $14 billion purchase of Karuna strengthens its CNS portfolio. Astellas Pharma's partnership with AviadoBio emphasizes the focus on gene therapies for neurodegenerative diseases. These trends underscore the importance of strategic investments and collaborations to drive innovation and meet market demands.

Regulatory Updates

Recent regulatory updates aim to improve transparency and efficiency in healthcare. The EU's revised CTIS rules enhance clinical trial data access, the FDA's Biosimilars Action Plan promotes biosimilar adoption, and new EU guidelines require environmental risk assessments for pharmaceuticals to protect ecosystems.

1. **Revised transparency rules for the Clinical Trials Information System (CTIS) were implemented, enhancing public access to clinical trial data in the EU**

   Effective June 18, 2024, the updated CTIS transparency rules expand public access to clinical trial data, benefiting patients, healthcare professionals, and sponsors. About 4,000 trials are now accessible, with 500 added monthly. Key updates include removing the deferral mechanism, simplifying sponsor processes, and balancing transparency with protecting Commercially Confidential Information. Data from prior trials will also be made public. Support materials are available to help sponsors adapt to these changes.[10]

2. **FDA updated its Biosimilars Action Plan to enhance regulatory clarity, streamline development, and boost biosimilar adoption**

   In June 2024, the FDA updated its Biosimilars Action Plan (BAP) to improve biosimilar availability and adoption in the U.S. Building on the 2018 plan, the update focuses on enhancing regulatory clarity, refining review processes, and addressing labeling practices, such as prohibiting interchangeable status claims. The FDA emphasizes stakeholder involvement and global regulatory harmonization to promote accessibility and cost efficiencies in biosimilar development.[11]

3. **The EU's updated guidelines for Environmental Risk Assessments (ERAs) mandate pharmaceutical companies to assess and mitigate environmental risks from medicinal products, focusing on transparency, mitigation measures, and ecosystem protection**

   Effective September 1, 2024, the EU's new guidelines strengthen Environmental Risk Assessments (ERAs) for all human-use pharmaceuticals, requiring risk evaluation and mitigation for aquatic and terrestrial ecosystems. Companies must ensure transparency in their assessments, facing challenges such as data availability and regulatory complexity. Expert support is available to help with compliance and tailored mitigation strategies.[12]

   Recent regulatory updates focus on transparency, efficiency, and sustainability, significantly impacting pharma companies. The EU's revised CTIS rules improve clinical trial data access, while the FDA's updated Biosimilars Action Plan boosts biosimilar adoption. The EU's new Environmental Risk Assessment guidelines mandate ecosystem impact evaluations. These changes require quick adaptation for compliance and competitiveness.

Recent Drug Approvals: Advancing Patient Care

The pharmaceutical landscape continues to evolve with groundbreaking advancements, offering hope to patients with challenging medical conditions. Recent approvals by the FDA and EMA highlight significant progress in addressing diverse therapeutic needs, from rare genetic disorders to advanced cancers and chronic conditions. In 2024, the FDA approved 50 innovative drugs that had never been previously approved or marketed in the United States, commonly referred to as "novel" drugs.[18] Below are some of the key drugs approved during the last year.

Alhemo (Novo Nordisk) was approved by the EMA on 17 October 2024 for preventing bleeding in patients aged 12+ with haemophilia A or B with inhibitors. It contains concizumab, a monoclonal antibody that enhances coagulation by targeting TFPI. Alhemo demonstrated efficacy in reducing bleeding episodes in a 24–32-week study.[13]

Zepbound (Eli Lilly) is an FDA-approved in December 2024 injectable for weight management in adults with obesity or overweight-related conditions. Containing tirzepatide, it works as a GLP-1 receptor agonist and is used alongside a reduced-calorie diet and increased physical activity.[14]

Crenessity (Neurocrine Biosciences) received FDA approval in December 2024 for treating congenital adrenal hyperplasia (CAH) in patients aged 4+. It contains crinecerfont and is used with glucocorticoids to control androgen levels, addressing hormone imbalances in CAH.[15]

Inavolisib (Genentech), received FDA approval in October 2024 branded as ITOVEBI, is an FDA-approved kinase inhibitor for advanced breast cancer. It treats endocrine-resistant, PIK3CA-mutated, HR-positive, HER2-negative cancer in adults, used with palbociclib and fulvestrant after recurrence.[16]

Balversa (Erdafitinib), approved by the EMA on 22 August 2024, treats urothelial cancer in adults with FGFR3 mutations. This oral kinase inhibitor is for patients with unresectable or metastatic cancer, offering a new option after immunotherapy failure.[17]

About ANSEA

ANSEA is a global healthcare consulting firm with offices in Singapore and Switzerland, dedicated to improving patient health outcomes for public and private sector institutions. Our expertise spans Commercial Planning, Market Access, Health Systems Research, and Stakeholder Engagement. Leveraging a network of country experts, we provide innovative solutions and local insights to support clients worldwide.

References

1. *The changing landscape of precision medicine. (2023, January 23).*
2. *FDA. (2023, December 8). FDA Approves First Gene Therapies to Treat Patients with Sickle Cell Disease.*
3. *Lilly's Zepbound® (tirzepatide) superior to Wegovy® (semaglutide) in head-to-head trial showing an average weight loss of 20.2% vs. 13.7% | Eli Lilly and Company. (2023). Eli Lilly and Company.*
4. *Amgen. (2024, November 26). AMGEN ANNOUNCES ROBUST WEIGHT LOSS WITH MARITIDE IN PEOPLE LIVING WITH OBESITY OR OVERWEIGHT AT 52 WEEKS IN A PHASE 2 STUDY. Prnewswire.com; Cision PR Newswire.*
5. *Müller, T. D., Blüher, M., Tschöp, M. H., & DiMarchi, R. D. (2021). Anti-obesity drug discovery: advances and challenges. Nature Reviews Drug Discovery, 21(3), 1–23.*
6. *Ogbonmide, T., Rathore, R. (2023). Gene Therapy for Spinal Muscular Atrophy (SMA): A Review of Current Challenges and Safety Considerations for Onasemnogene Abeparvovec (Zolgensma).*
7. *Healthcare, G. (2024, February 8). Novo Nordisk to acquire CMO giant Catalent for $16.5bn. Pharmaceutical Technology.*
8. *Bristol Myers Squibb Completes Acquisition of Karuna Therapeutics, Strengthening Neuroscience Portfolio. (2020).*
9. *News | Astellas Pharma Inc. (2024, October 8).*

10. *Launch of revised CTIS transparency rules. (2024, April 22). Accelerating Clinical Trials in the EU.*
11. *Research, C. for D. E. and. (2024). Biosimilars Action Plan. FDA.*
12. *Committee for Medicinal Products for Human Use (CHMP) Guideline on the environmental risk assessment of medicinal products for human use. (2024).*
13. *Alhemo | European Medicines Agency (EMA). (2024, October 18). European Medicines Agency (EMA).*
14. *Zepbound (tirzepatide) Injection for Adults with Obesity or Excess Weight. (2024). Zepbound.lilly.com.*
15. *Office. (2024). FDA Approves New Treatment for Congenital Adrenal Hyperplasia. U.S. Food and Drug Administration.*
16. *Genentech: ItovebiTM (inavolisib) - Information for Patients. (2019). Gene.com.*
17. *Balversa (erdafitinib). (2024).*
18. *Novel Drug Approvals for 2024 | FDA*