Ultra High-Cost Drugs

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Key takeaways

• Ultra high-cost drugs (UHCDs) are expensive, potentially durable, or curative therapies currently approved to treat conditions that are orphan and rare disease.

• UHCDs are relatively new pharmaceutical products in the market, but their presence will almost certainly increase dramatically over the next decade.

• UHCDs are disrupting three elements of healthcare: care delivery, generation of clinical evidence, and cost/payment models.
Ultra high-cost drugs (UHCDs) are expensive, potentially durable, or curative therapies currently approved to treat conditions that are orphan and rare disease.

Home tests – also called self-tests or home-use tests – are typically sold over the counter and allow users to test self-collected specimens and interpret the results on their own without the help of trained health professionals.

Biomarker testing (also known as mutation, genomic, or molecular testing) uses laboratory tests to help the health care team gather as much information as possible about a patient’s disease state.

Deep Brain Stimulation (DBS) is the implantation of a device that sends a signal to brain areas responsible for body movement to improve the lives of patients with Parkinson’s, other neurodegenerative diseases, and behavioral and mental health disorders.

Pharmacogenomics (PGx) is the science of understanding the influence of a person’s genome (i.e., their complete set of DNA and genes) on drug treatment outcomes.

Psychedelic-assisted therapy entails using a high dose of a psychedelic substance to treat a variety of behavioral and mental health issues such as addiction, mental health conditions.
What is it?

Ultra high-cost drugs (UHCDs) are potentially expensive, durable, or curative therapies and serve to treat conditions that are often orphan and rare diseases. UHCDs, which are (exclusively at the moment) comprised of gene and cell therapies, promise durable effects – at least 15 years. Unlike other expensive specialty drugs that help patients manage chronic conditions, ultra-high-cost drugs promise long-term amelioration of the condition after a single application. Up to now, these gene and cell therapies have largely targeted orphan and ultra-orphan diseases. But over the next decade a wave of these drugs focused on more common conditions will enter the market with the potential to impact millions of Americans.

UHCDs are typically extremely expensive, ranging from thousands to millions of dollars. The cost of UHCDs is often justified as they typically require very few applications and could displace other longitudinal care. However, while they promise long-term savings by alleviating much of the physical burden of the illness, their high upfront cost can hit purchasers and consumers hard.

To mitigate against the financial risk for novel therapies, purchasers of these products are exploring new financing models with manufacturers. Performance-based agreements and orphan reinsurance benefit managers are gaining traction.

Some of the better-known examples of UHCDs currently on the market are Luxturna and Zolgensma, which treat Leber Congenital Amaurosis (LCA) and Spinal Muscular Atrophy (SMA) respectively, Danyelza to treat neuroblastoma, Solris to treat atypical hemolytic uremic syndrome, and Yescarta CART-cell therapy for non-Hodgkin lymphoma. In addition, it is also likely that gene therapies for Hemophilia and Sickle Cell will enter the market in the next three years. These treatments are indicated for much larger populations than LCA and SMA.

Source: Advisory Board interviews and analysis.
Why does it matter?

Ultra high-cost drugs are relatively new pharmaceutical products in the market, but their presence will almost certainly increase dramatically over the next decade. Current estimates indicate that there will likely be around 60 by 2030. Not only will there be more of these durable treatments in the next decade, but many hitting the market will treat more prevalent illnesses than those currently available. There is likely to be a durable therapy launching in 2023 for the treatment of hemophilia A in adults, which at only 30% market penetration would still cost plans hundreds of millions of dollars. Health plans have a limited window to prepare themselves for this challenge, which while present today, will only grow in the coming decade.

Three forecasts for gene and cell therapy spending through 2026

*In millions of dollars*

What makes it disruptive?

Areas of disruption

Care delivery

The nature of orphan and rare diseases pose diagnostic and treatment challenges. Clinicians often don’t have adequate knowledge on rare conditions treated by some UHCDs, and as a result patients may not be aware of what they have, and subsequently how it can be treated.

Clinicians need to think differently about how they care for all patients, because they may not know if a patient presenting with an issue actually has one of these now treatable orphan or rare diseases. Clinicians also need to increase their awareness of treatment and administration options, especially as UCHDs are not administered at all hospital settings.

Additionally, there has been disruption in the delivery system, with the formation of nationwide pharmacies specializing in these UHCDs. Pharmacies must be equipped to manage these drugs.

Clinical evidence

UHCDs promise long-term benefits, but to prove these benefits will require major changes in the way clinical evidence is generated. Currently, UHCDs are approved based on traditional stage 3 clinical trials that last only about 18 months. However, this evidence cannot fully predict whether these products will actually deliver on promised durability. Payers, providers, and manufacturers are all looking to innovate in how they can gather real world evidence (RWE) in a systematic way to support claims for the durability of these drugs.
What makes it disruptive?

Cost/payment models
Payment models for these drugs are an evolving landscape. UHCDs are extremely expensive, ranging from thousands to millions of dollars, and the cost of these drugs are justified by manufactures by the annual cost of care they are replacing and the impact on survival and quality-adjusted life years.

Not only are payment models for these drugs evolving, but it is also clear that traditional payment models won’t work. These high costs raise the need for new payment models that often tie total compensation to longitudinal performance against mutually agreed upon end points. Thus, new payment models are necessary for adoption.

A note on health equity
People of color are disproportionately impacted by chronic illnesses and certain health conditions. Because high drug prices disproportionately affect low-income, uninsured, and people of color, state laws that work to lower drug costs for these communities are pivotal in improving access and health equity.

Additionally, utilizing representative data, breaking down barriers to identifying people from marginalized groups who may not have access, trust, and awareness can improve health disparities.
## Conversations you should be having

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<th>Sector</th>
<th>Conversations</th>
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<td><strong>Payers</strong></td>
<td>• How can my organization develop financial solutions around UHCDs to help mitigate financial risks associated with these novel therapies?</td>
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<td>• How do we make UHCDs accessible to the targeted populations by collaborating on risk-mitigating contracts with manufacturers?</td>
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<td>• How can we structure reimbursement to account for performance risk?</td>
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<td><strong>Health systems</strong></td>
<td>• How can we educate those within our organization about specialty pharmacy terminology and key issues surrounding the therapeutic areas treated by UHCDs?</td>
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<td>• What guidelines can be implemented to create cross-continuum care management? Which standards can be improved upon? (patient identification, outreach, enrollment, and graduation guidelines)</td>
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<td>• How can we minimize financial risk to the health system?</td>
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<td>• How can we support identification and financial access for eligible patients?</td>
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<tr>
<td>Pharma</td>
<td>• How can we utilize and harmonize representative data across multiple trials, to prove adequate safety and efficacy?</td>
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<td>• How do we collaborate with clinicians from various organization types to anticipate and mitigate challenges they will face, including data collection to support performance-based contracts?</td>
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<td>Cross-sector</td>
<td>• Which areas are optimal for collaboration and partnership, to decide specialty drug prescribing, sourcing, administration, and reimbursement?</td>
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<td>• How can my organization educate providers to support patient identification and appropriate treatment?</td>
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<td>• How can we ensure appropriate distribution and administration channels?</td>
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<td>• What strategies can we implement to track patient outcomes over time and generate evidence clarifying long-term outcomes?</td>
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  Read now
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