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Transforming Global Healthcare: Establishing a Collaborative Framework for Specialty Pharmaceutical Stakeholders in the Treatment of Complex, Chronic Diseases

AscellaHealth

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INTRODUCTION

The global healthcare landscape is undergoing a transformational shift as the prevalence of complex, chronic diseases (CCDs) surges, causing the demand for specialty pharmaceuticals to increase rapidly. Today, over 300 million people live with a complex condition or rare disease worldwide, affecting 3.5% – 5.9% of the global population; numbers that are projected to grow steadily. At the same time, healthcare spending continues to expand, with specialty drugs accounting for a disproportionately large percentage of total pharmaceutical expenditures relative to prescription rates.

These trends underscore the critical challenges pharmaceutical industry stakeholders encounter in balancing the potential of life-saving treatments with the escalating costs and logistical complexities associated with their delivery. This white paper explores some of the most pressing issues facing life sciences companies, payers, healthcare providers and, of course, patients, analyzing effective ways to improve access to treatments, support better clinical outcomes, manage rising costs, and streamline the commercialization process. With a specific focus on the innovative strategies employed by industry leaders, we seek to understand how collaborative, multifunctional solutions can pave the way for more sustainable and effective healthcare for patients around the world.

BRIDGING THE GAP IN UNDERSTANDING COMPLEX, CHRONIC DISEASES

Managing complex, chronic diseases (CCDs) presents unique and substantial challenges due to their sophisticated nature and the extensive coordination required for effective care. Patients frequently contend with multiple health conditions, navigate interactions with many different healthcare providers, and often struggle to make informed decisions because of insufficient access to useful information. It is vital to understand the scope of CCDs and the pivotal role of specialty pharmaceuticals in managing them to build toward a more efficient ecosystem.

Chronic disease typically persists for one year or longer, often involves coexisting health conditions, and necessitates continuous treatment and management —often without spontaneous improvement. While diseases like hepatitis B or C, multiple sclerosis, and rheumatoid arthritis are more common and well-known, there are also many other lesser-known conditions, including hereditary angioedema, Pompe disease, and Cushing's syndrome, that are starting to become more widespread.

The growing demand for specialty pharmaceuticals to treat these conditions has sparked an influx of investment dollars and fueled the development of progressive new therapies, offering hope where there previously was none. Similarly, with a focus now shifted toward innovation, providers are aiming to align treatments with appropriate care settings, develop diverse strategies to address a wide range of patient needs and ultimately enhance proactive cost management methods. This paradox of championing innovation versus mitigating financial burden presents a significant hurdle for healthcare stakeholders, requiring unique approaches to find an equitable balance between access and affordability.

Some Specialty & Rare Disease States

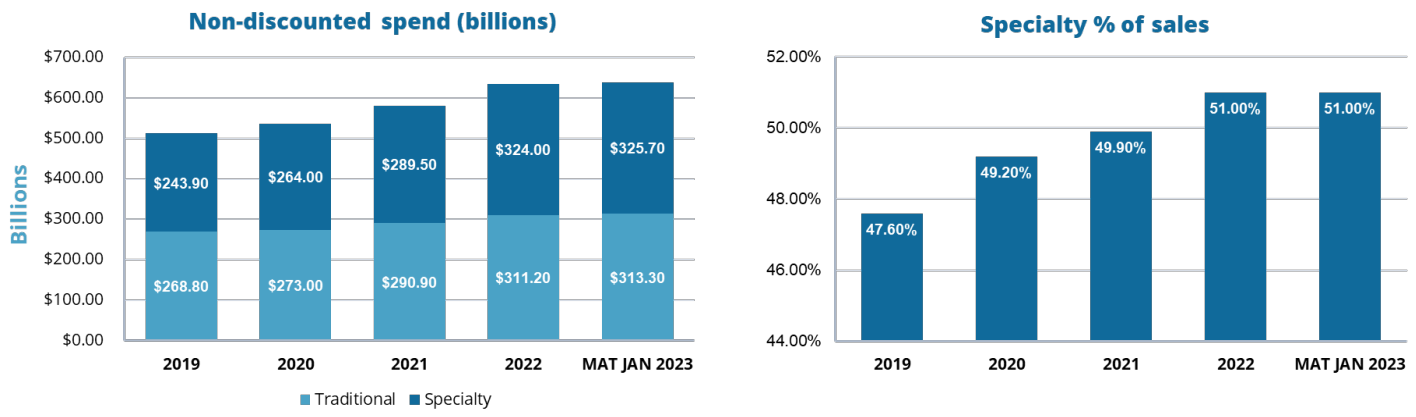
- Alkaptonuria
- Ankylosing Spondylitis
- Cancer
- Crohn's Disease
- Cushing's Syndrome
- Cystic Fibrosis
- Duchenne Muscular Dystrophy
- Eosinophilic Esophagitis
- Fields Condition
- Harlequin-Type Ichthyosis
- Hemophilia
- Hepatic Encephalopathy – Xifaxan®
- Hepatitis B and C
- Hereditary Angioedema
- Hidradenitis Suppurativa
- HIV-PrEP
- Hutchinson-Gilford Progeria
- Hyperlipidemia
- Idiopathic Thrombocytopenia
- Juvenile Idiopathic Arthritis
- Kuru Disease
- Multiple Sclerosis
- Niemann-Pick Disease Type C
- Phenylketonuria (PKU)
- Pompe Disease
- Propionic Acidemia
- Psoriatic Arthritis
- Recessive Dystrophic Epidermolysis Bullosa
- RPI Deficiency
- Severe Combined Immune Deficiency (SCID)
- Systemic Lupus Erythematosus/ Lupus Nephritis
- Tyrosinemia
- Ulcerative Colitis
- Uveal Melanoma

NAVIGATING THE COMPLEXITY AND COST OF SPECIALTY PHARMACEUTICALS

To fully grasp the impact of specialty pharmaceuticals within the healthcare ecosystem, it is important to examine both the logistical and financial implications, as the increased use of these medications in the treatment of CCDs introduces a new layer of complexity and cost. Often requiring specialized handling and delivery, dosing supervision, administration and monitoring, these drugs are typically not made available through traditional retail pharmacies. Likewise, dedicated patient support becomes paramount because of the intricacies involved, various delivery methods -- such as injections or infusions -- and specialized requirements. Comparatively speaking, this experience is much different from that of routine prescription medications.

While advanced therapies have emerged as a cornerstone in the treatment of chronic diseases, their characteristically high costs can render them unsustainable; for instance, treatments for a condition like rheumatoid arthritis may exceed tens of thousands of dollars annually per patient. Most often, these types of medications are administered in extended therapy cycles with an intended treatment duration of over six months. The burden of these high-cost medications is not only felt by healthcare payers and patients, who may face significant out-of-pocket expenses, but also manufacturers who contend with the issues of inaccessibility, sub-optimal patient experiences and poor compliance.

US Specialty Spend Outpaces Traditional Drugs

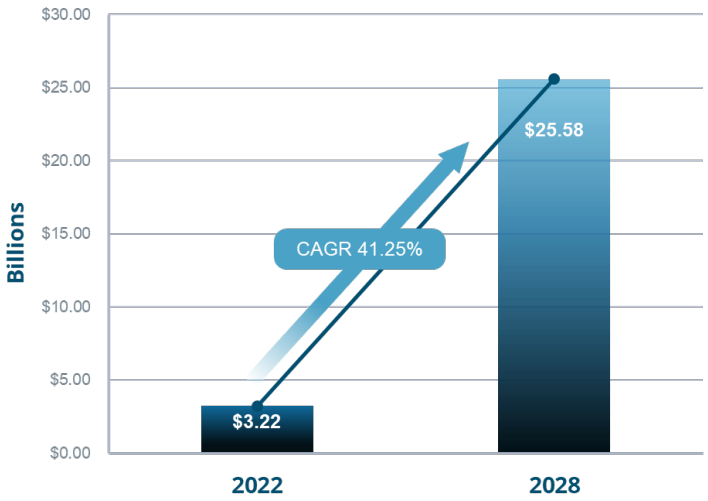


Source: IQVIA https://www.iqvia.com/locations/united-states/blogs/2023/07/insights-into-the-2023-us-pharmaceutical-market#:~:

Despite facing these obstacles, it is crucial to continue seeking cutting-edge solutions. The financial pressures of existing specialty drugs highlight the urgent need for advanced therapies that can offer long-term value for their stakeholders. This pursuit also lays the groundwork for evaluating emerging advancements such as cell and gene therapies (CGTs). Although these treatments bring their own set of challenges, they represent the pinnacle of medical innovation targeted at addressing the underlying root causes of CCDs.

US Cell & Gene Therapy Market

- The US Cell and Gene Therapy market was valued at $3.22 billion in 2022 and is projected to reach $25.58 billion in 2028, with a compound annual growth rate (CAGR) of 41.25%, during the study period.
- Emerging Segment: Genetic disorders anticipate the fastest growth due to rising cases.
- **CGT comprised 10% of all U.S. FDA novel approvals in 2023.**



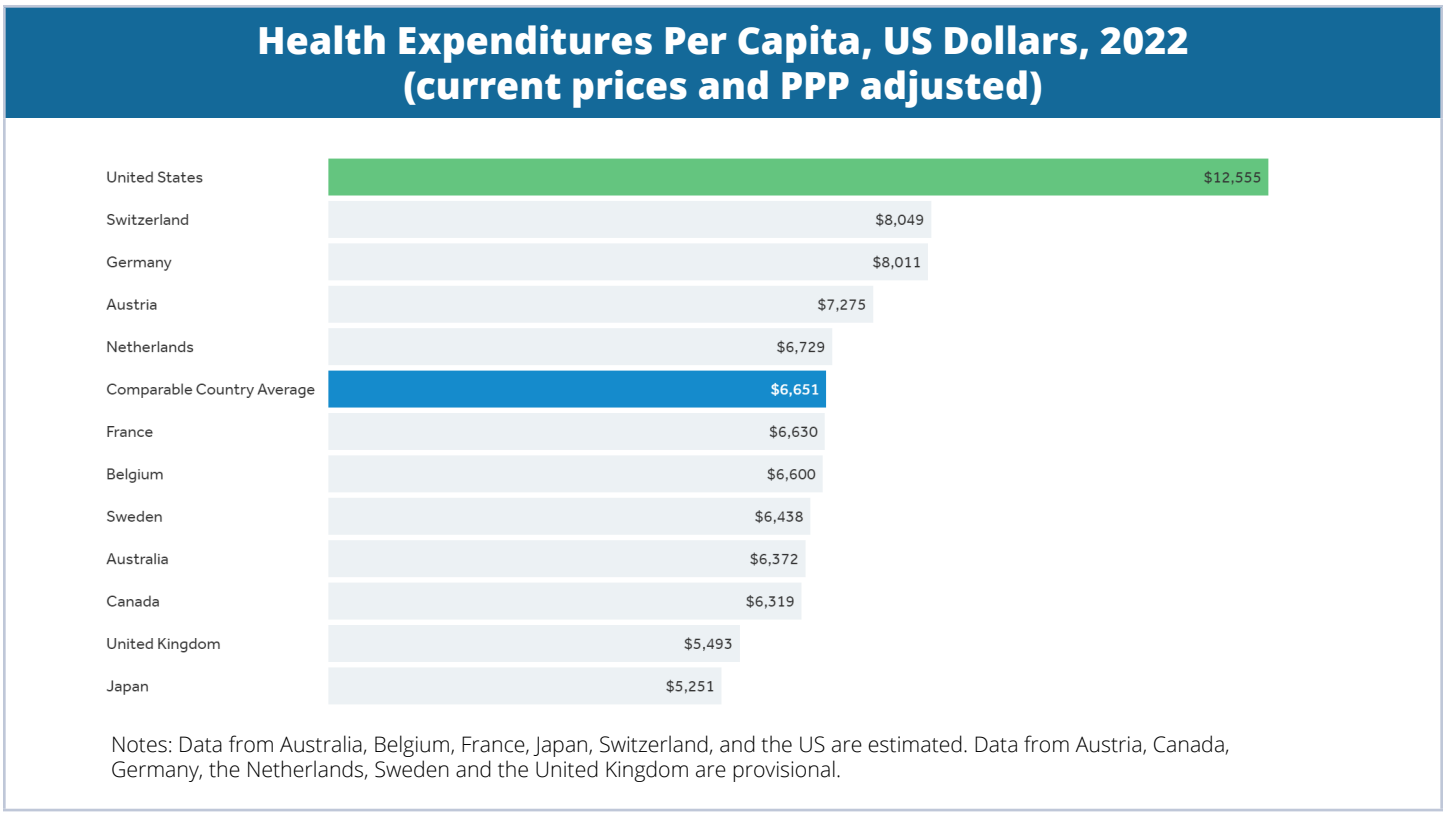
Source: https://www.researchandmarkets.com/reports/5806791/us-cell-and-gene-therapy-market-focused-insights?utm_source=BW&utm_medium=PressRelease&utm_code=tw7bgr&utm_campaign=1861598+-+US+Cell+%26+Gene+Therapy+Market+Report+2023-2028%3a+Industry+Expected+to+Grow+at+a+CAGR+of+41.25%25&utm_exec=shbe20prd

GLOBAL HEALTHCARE SPENDING – A FOUNDATION FOR SPECIALTY DRUG EXPENDITURES

A more precise understanding of global healthcare spending provides a foundation for identifying the investment in breakthrough specialty drugs and therapies launched in recent years to address multiple CCDs.

Healthcare spending is at an all-time high, with health expenses representing a growing share of gross domestic product across the board. Since 2000, the price of medical care, including services provided as well as insurance, drugs and medical equipment, has increased by 114.3%. In contrast, prices for all consumer goods and services rose by 80.8% in the same period. Unfortunately, many American families have seen the costs of health services and premiums grow faster than their wages.

Comparing health spending in the US to other countries is complex, as each country has distinct political, economic and social attributes contributing to its spending. Wealthy countries, including the US, tend to spend more per person on health care than lower-income countries.



Source: https://www.healthsystemtracker.org/chart-collection/health-spending-u-s-compare-countries/#Health%20expenditures%20per%20capita,%20U.S.%20dollars,%20PPP%20adjusted,%202022

Breakdown of Specialty Pharmaceutical Spending

Pharmaceutical expenditures in the US now exceeds $600 billion annually, and according to reports in 2022, specialty drugs represented 27% of overall spending and 0.7% of claims -- with spending per plan member for specialty drugs increasing by 4%. Utilization, the proportion of plan members making a specialty claim, increased by 5.4%.

On a global level, robust purchasing capabilities and extensive reimbursement support for specialty pharmaceuticals are expected to drive growth. By 2027, specialty medicines are projected to represent about 43% of global spending, including 56% of total spending in developed markets. Novel cancer drugs are leading this trend and are expected to reach $370 billion by 2027.

CGTs have also been steadily gaining momentum. And while these treatments are a testament to the incredible advancements in biotechnology, they are also significant contributors to the skyrocketing expenditures within the global healthcare ecosystem.

Spotlight on Cell and Gene Therapies

The introduction of new CGTs approved in the past three years is a primary source of elevated spending and is projected to increase the median price of treatment in 2024 and beyond. As of August 2024, 38 gene therapies have been approved. There are an additional 500 in the pipeline, and the expectation is that 10–20 will be approved annually by 2025. In the US, more than 600 gene therapies are in clinical trials, and it's estimated that more than 1 million Americans will be treated with gene therapy by the end of 2034, accounting for about $25 billion annually.

In 2023, the industry saw the approval of the first CRISPR-Cas treatment for sickle-cell disease by the UK and US, with staggering costs of $2-3.1 million per person. In 2024, the US approved Lenmeldy, a gene therapy for children with a rare genetic disease, and it is now the most expensive drug in the US at $4.25 million.

Cell & Gene Therapies In Market

- An increasing number of promising CGTs are currently in development for both orphan and high prevalence diseases.
- While CGTs have the potential to be curative, they come with a substantially greater upfront cost.

	Drug Name	Indication	AWP
casgevy	Casgevy®	Sickle Cell Disease	$2,640,000
KYMRIAH	Kymriah®	Follicular Lymphoma	$512,457
LUXTURNA	Luxturna®	Inherited Retinal Disease	$510,000
lyfgenia	Lyfgenia	Sickle Cell Disease	$3,720,000
ROCTAVIAN	Roctavian	Hemophilia	$2,900,000
YESCARTA	Yescarta®	Non-Hodgkin's Lymphoma	$554,400
zolgensma	Zolgensma®	Spinal Muscular Atrophy	$2,786,453
zynteglo	Zynteglo®	Beta Thalassemia/ Cooley's Anemia	$3,360,000

SLOWING DOWN SPECIALTY SPEND

Payers are responding to the exorbitant cost of novel specialty therapies and applying more thorough assessments of the cost/benefit of therapies in their coverage decisions. Financial solutions are under development as payers seek to tie reimbursement to clinical outcomes and real-world evidence, enacting prior authorization and other controls to manage specialty drug utilization.

One highly effective cost-saving solution is Site of Care (SOC) optimization for infusion therapies. By transitioning patients from higher-cost hospital settings to less costly alternatives such as freestanding physician offices or ambulatory infusion centers, SOC optimization helps reduce unnecessary healthcare spending without compromising the quality of care. Comparatively speaking,

drugs administered outside of hospital settings are, on average, $1,400 less per treatment, while hospitals are charging double the price of what specialty pharmacies charge for the same drugs.

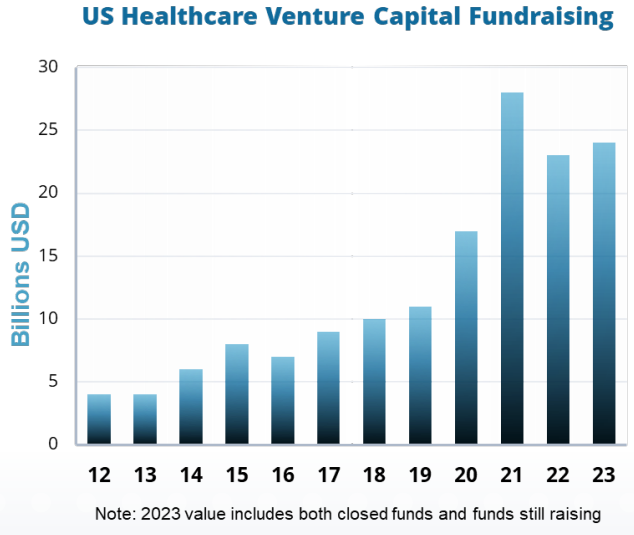
Another method that can help cut costs is the usage of biosimilars. This approach has saved a reported $9.4 billion in spending. Biologic drugs are very similar to already approved "reference" biologics in potency, safety and efficacy, but different companies manufacture them. The sales price for biosimilars averages 50% less than the reference brand biologic price at the time of biosimilar launch.

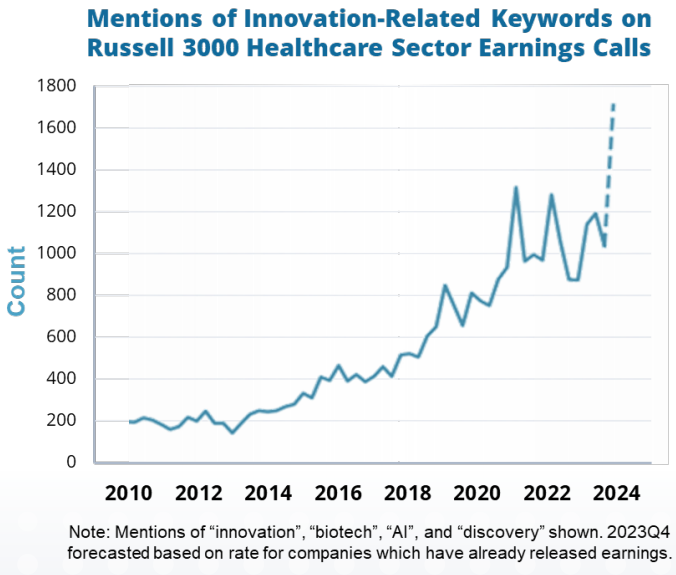
FACTORS DRIVING ROBUST, SUSTAINED MARKET GROWTH

Despite the high cost and access issues, the specialty pharmaceutical market is experiencing continued growth driven by several key factors:

- **Growing Need for Specialty Pharmaceuticals:** The amount of people living with a complex or rare disease across the world now reaches into the hundreds of millions and the ability to effectively treat them has become increasingly difficult. These conditions are often chronic, progressive and debilitating and can lead to significant morbidity and mortality.
- **Increased Investment:** The specialty pharmacy market is projected to continue growing by 8% per year through 2025, fueled mainly by new-to-market drugs. The increase in capital investments paves the way for creating innovative specialty pharmaceuticals, technology and CGTs, as personalized medicine and biologics are dramatically impacting market expansion.

Healthcare Innovation Investor Interest





Source: GS Data Works, SVB, Goldman Sachs Global Investment Research

- **Increased Access to Information:** Supplementary education and support programs are vital for driving growth in the specialty pharmaceutical market. By improving patients' and caregivers' understanding of complex conditions and therapy options, treatments can be tailored more accurately and managed successfully, leading to a better product experience and increased market demand.
- **Sustainable Pricing Models:** Various financial arrangements are emerging for different therapies and treatments, including financial risk-based contracts, health outcomes contracts, mortgage models, subscription models, indication-specific pricing and volume-based purchasing.
- **Government Regulations and Expedited Approval Pathways:** It's essential to initiate the market access process early in the product lifecycle, ideally during Phase II or III clinical trials. Doing so ensures that a product meets regulatory requirements and gains payer approval within a defined timeframe, accelerating patient access to treatment. Regulatory agencies worldwide recognize the increased urgency and are speeding up the approvals of specialty drugs that treat complex diseases. For example, the FDA has developed four distinct and successful approaches to making them available as quickly as possible.

Fast Track

Fast-track is a process designed to facilitate the development and expedite the review of drugs that treat serious conditions and fill an unmet medical need.

Breakthrough Therapy

This is a process designed to expedite the development and review of drugs which may demonstrate substantial improvement over available therapy.

Accelerated Approval

These regulations allow drugs for serious conditions that fill an unmet medical need to be approved based on a surrogate endpoint.

Priority Review

A Priority Review designation means the FDA aims to act on an application within six months.

Source: https://www.fda.gov/patients/learn-about-drug-and-device-approvals/fast-track-breakthrough-therapy-accelerated-approval-priority-review

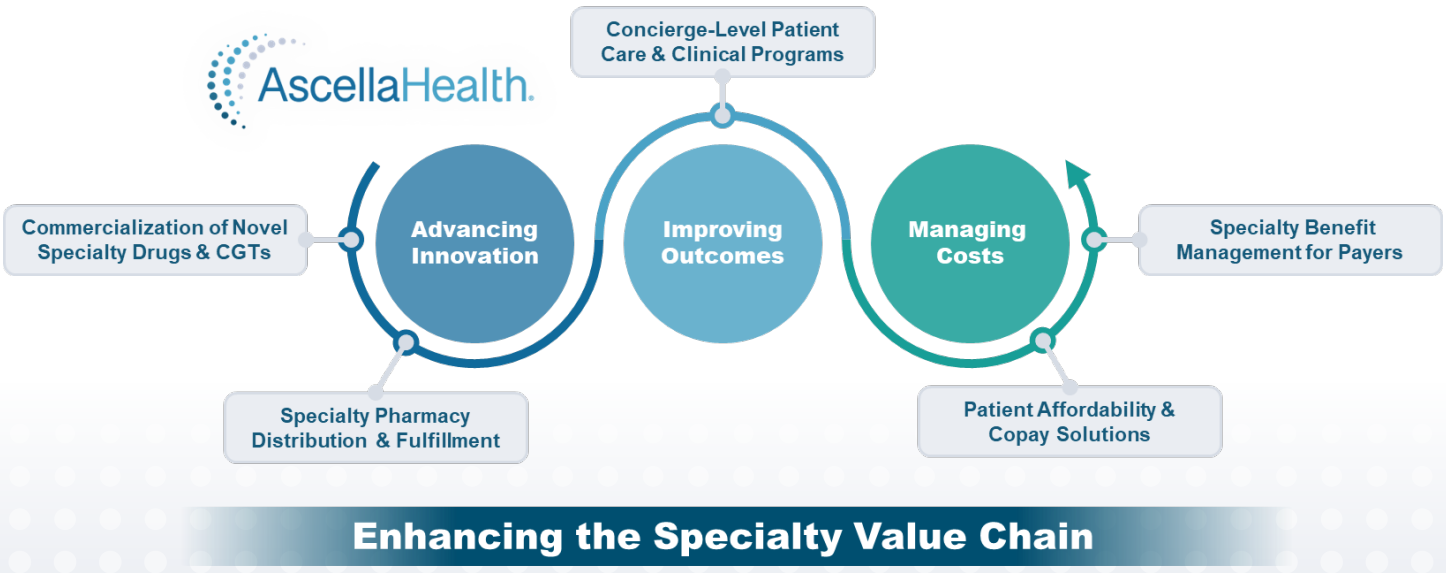
ASCELLAHEALTH: DRIVING STAKEHOLDER COLLABORATION AND HEALTHCARE INNOVATION

AscellaHealth is spearheading the transformation of healthcare delivery by fostering collaboration among stakeholders and driving forward innovative solutions. In an industry where the complications of commercializing specialty pharmaceuticals compound the challenges of managing CCDs, AscellaHealth embraces a cooperative approach as a model for how effective partnerships can glean efficiency in framing and executing effective market access programs.

Central to AscellaHealth's strategy is its commitment to integrating the interests of all vested parties—patients, healthcare providers, payers, and pharmaceutical manufacturers. As a bridge between these groups, AscellaHealth ensures that each party's needs are met while keeping the patient at the center of care. This careful coordination enables the joint development of tailored solutions; solutions that address the specific issues centered around the treatment of chronic diseases, promoting broader access to specialty drugs and carefully managing the financial obligations associated with proper care.

AscellaHealth is actively redefining the standards for managing pharmacy costs for self-insured groups, captives, at-risk providers, ACOs, PBMs and regional health plans. These widely adaptable and scalable pharmacy management solutions include specialty and medical cost management, formulary and network design, custom clinical programs and data analytics to assess and maximize health outcomes. By leveraging real-time data, AscellaHealth can identify trends, isolate gaps and target interventions to improve efficiency.

AscellaHealth's approach to stakeholder collaboration and innovation not only addresses the immediate challenges of managing CCDs but also sets the stage for long-term advancements in healthcare.



Enhancing the Specialty Value Chain

Case Study Example: A Model of Pharma & Payer Collaboration

In the rapidly evolving landscape of specialty pharmaceuticals, effective collaboration between pharmaceutical companies and payers is crucial for ensuring patients receive the right treatments in a timely manner. A prime example of this approach is evidenced in the partnership between AscellaHealth and a pharmaceutical manufacturer specializing in the rare disease sector.

This manufacturer focuses on developing innovative therapies that can potentially treat patients with a wide variety of serious disorders. Through their research and development efforts, they have discovered more than 1,000 proprietary compounds and conducted advanced clinical trials for numerous chronic conditions. Their groundbreaking work led to the introduction of a first-of-its-kind treatment for a rare endocrine disorder, approved by the FDA, marking a significant milestone.

Recognizing the complexities of the distribution process and the need for comprehensive patient support to successfully bring their new, breakthrough drug into the market, the manufacturer partnered with AscellaHealth to improve communication flows, optimize their product distribution, and enhance the patient journey from start to finish. This partnership extended well beyond traditional service-provider relationships, combining expertise from various healthcare stakeholders and advocacy resources with targeted analytics to improve patient care processes, support payer services, and inform market access and strategic planning activities—finding a way to navigate the complexities and costs associated with CCDs and specialty pharmaceuticals.

AscellaHealth's Role as an Exclusive Specialty Pharmacy

Personalized Patient Support Services

AscellaHealth provides comprehensive HUB services that streamline the patient journey, managing every aspect of care while collaborating closely with patients and HCPs. Dedicated care teams guide patients through enrollment and reimbursement processes, ensure timely access to prescriptions and provide education to help patients understand and follow their course of therapy.

Additionally, AscellaHealth patient care teams cohesively connect patients with their Pharmacists and HCPs to promote better adherence to therapy, monitor for health changes and promptly address issues with their course of treatment.

Payer Services

AscellaHealth collaborates proactively with the patient's insurance company and HCP to address clinical coverage criteria and prior authorization. By validating the clinical rationale for the product, AscellaHealth supports efforts to confirm insurance coverage. Data and information are shared with payers to streamline prior authorization processes.

AscellaHealth lowers patient out-of-pocket costs through manufacturer copay programs and patient assistance programs (PAPs) for those individuals who meet financial requirements. It also ensures patients have sufficient medication during the prior authorization process and renewal periods, providing each patient with a "starter" and/or "bridged therapy" to assure a seamless transition and uninterrupted therapy.

Benefits and Key Differentiators of the Exclusive Partnership Model

The AscellaHealth exclusive distribution model includes limited drug distribution and provides white glove, high-touch services that result in more efficient processes. Ensuring a seamless, coordinated solution, AscellaHealth helps reduce cost inflation while enhancing quality of care.

Enhanced Patient, HCP and Payer Engagement:

- **High-Touch Services:** Personalized interactions with patients and HCPs improve therapy adherence and patient outcomes. This level of service leads to greater efficiency and efficacy throughout the patient journey -- from the initial prescription to coordinating the timely delivery of therapy.
- **Educational Opportunities:** Engaging directly with prescribers and patients allows AscellaHealth to understand the specific needs of the patient and their management of care. This coordination enables the development of targeted educational resources that support more informed decision-making and enhance the overall patient experience.
- **Reliable, Single-Source Solution for Payers:** As the exclusive pharmacy, AscellaHealth collaborates directly with payers who become familiar with our policies, quality of service and identification of key performance indicators (KPIs). Payers that utilize a sole-source provider for patient care coordination traditionally see better results and greater efficiencies in therapy management.

A VISION FOR THE FUTURE: DRIVING INNOVATION FORWARD

As we look forward, it's clear that the shared vision of a more patient-centered, cost-efficient, accessible future in healthcare will be defined by our collective ability to pursue and sustain successful partnerships. Given the increased focus on CCDs worldwide, AscellaHealth is optimistic that the global specialty pharmaceutical industry will continue to develop and thrive with the integration of cross-functional strategies. The quarterly publication of AscellaHealth's Specialty & Rare Pipeline Digest™ validates the increase of groundbreaking therapies, and provides timely updates into specialty product pipelines including new, pending and upcoming Specialty and Rare Disease drug launches, CGTs, and biosimilars.

We encourage stakeholders throughout the industry to engage in meaningful discussion and emphasize working in partnership to overcome some of humanity's most intractable diseases. Together, we can drive innovation forward to ensure the best possible clinical outcomes for patients and pave the way for more sustainable specialty therapy programs. For more information about how AscellaHealth is contributing to these efforts and to explore potential partnerships, please visit our website or contact businessdevelopment@ascellahealth.com.

ABOUT ASCELLAHEALTH

AscellaHealth is a global partner that delivers proven end-to-end solutions to both life sciences and healthcare companies to enhance quality of life for patients with complex, chronic conditions. Every day our team gets critical healthcare products from manufacturers to patients while ensuring an efficient flow of funds between payers and pharma.

AscellaHealth partners with life sciences manufacturers around the world, enabling them to successfully commercialize therapies for complex, chronic conditions. Our comprehensive suite of services guides clients through every stage of the process, from clinical trials through approval, pre-commercialization support and ultimately transitioning patients on to therapy.

Our global expertise in specialty fulfillment, data analytics and patient support/HUB services allows us to streamline product launch, provide an ecosystem of financial support to our partners and ensure patients have access to therapies they need for better outcomes.

Visit www.ascellahealth.com

1055 Westlakes Dr., Suite 200
Berwyn, PA 19312
877.389.9040
info@ascellahealth.com

AscellaHealth's Specialty Pharmacy is located in St. Louis, MO. AscellaHealth Europe is comprised of locations in Dublin, Ireland and Manchester, England.

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