Insightsbriefing

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Managing the Thriving Specialty Pipeline

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Of the 46 new drugs approved in 2017, 39% were for orphan indications.

The U.S. drug pipeline continues to be robust. By one estimate, nearly 7,000 medicines are currently in development — almost three-quarters of them potentially first-in-class.[1] Between now and 2020, 533 new drugs are expected to seek marketing approval from the U.S. Food and Drug Administration (FDA), according to our analysis.* Of these, 291 (55 percent) are specialty drugs. This pipeline activity is contributing to the continuously expanding importance of the specialty drug market, which, it is estimated, will grow from $195 billion in 2016 to $280 billion in 2021.[2]

The numbers are only part of the story. Scientific developments are altering the pace and focus of drug development, while regulatory changes are shortening approval times and, in some cases, reducing the level of evidence required prior to drug approval. These marketplace realities pose new challenges for payors in their efforts to maintain patient access while keeping specialty cost and trend in check.

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Record FDA Approvals

The past year was an exceptional time for drug approvals. The FDA approved 46 novel medications in 2017 — more than double the 2016 figure, and the highest number of approvals in more than two decades. One-third of these new drugs were first-in-class, and 37 percent were designated breakthrough drugs, up from 32 percent in 2016 and 22 percent in 2015.[3, 4]

Part of the quicker pace of approvals has to do with a renewed focus at the agency on reshaping the drug approval process to expedite the availability of therapies that show dramatic results in early clinical studies.[5] Consistent with this approach, the agency is encouraging manufacturers to use its various pathways for accelerated review, which last year accounted for more than 60 percent of approvals, and to make greater use of surrogate endpoints, which can reduce the length of clinical trials by years.[3, 6]

The FDA also accelerated the approval process for generics, approving a record 1,027 in 2017 and took steps to encourage the development and use of biosimilars.[7] But the path to market for these competitive options — especially biosimilars — has been slow and difficult. There are many examples of brand manufacturers using contracting and aggressive patent protection strategies to keep competitors at bay. For example, Humira, the world's best-selling drug, lost its main U.S. patent in 2016.[8] Two biosimilars have been approved, but neither has reached the market, and the first-approved is currently not expected to be sold in the U.S. prior to 2023.[9] Similar issues are delaying the launch or obstructing the uptake of biosimilars for Neupogen and Herceptin, among others.[10, 11]

1,027

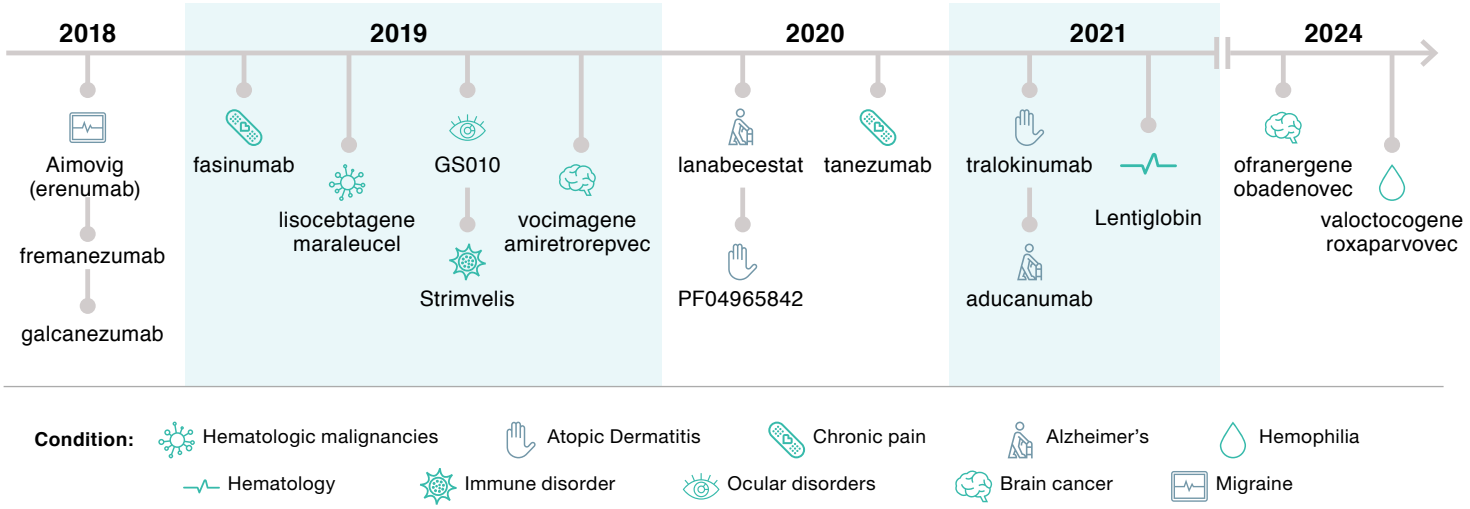
A record number of generics were approved in 2017.

Scientific Advances

What is striking about the pipeline today is the preponderance of drugs with new mechanisms of action and that, increasingly, challenge the very concept of conventional pharmacologic therapy.

New, just-emerging approaches to therapy promise profound change for some patient groups. Last year the FDA approved the first-ever gene therapies that use genetic engineering to replace mutated genes, effectively treating, and potentially curing, the patient's medical condition. Gene therapies launched so far have been expensive and the cost trend will likely continue. A course of therapy with Luxturna, which treats a form of inherited vision loss, costs $850,000.[12] These agents are complex and highly specialized and can be administered only by a limited number of qualified treatment centers. Some have the potential to replace years of treatment with drugs that cost hundreds of thousands of dollars annually and fundamentally change how previously incurable and/or untreatable conditions are managed.

Robust Pipeline of Drugs with Novel Mechanisms



Populations

Rare diseases continue to play an ever-increasing role in the drug pipeline. Of the 46 new drugs approved in 2017, 18 were approved for orphan indications — diseases that affect 200,000 or fewer U.S. patients. And if supplemental indications for already-approved drugs are included, the agency approved a record-breaking 77 therapies for orphan indications last year.[3, 13] Drugs for rare diseases have come to be a significant part of the pharmaceutical marketplace. By 2022, 21.4 percent of global prescription drug sales (excluding generics) will be for rare diseases, by one estimate.[14]

Equally striking is the emergence of several biologic therapies that are not intended to treat orphan or rare conditions. Today, complex biologic treatments are increasingly being brought to market for diseases with much larger patient populations, such as migraine, asthma, and arthritis, but still with comparably high list prices. This means their impact on trend is growing exponentially, forcing payors to find new strategies for dealing with them.

> Orphan drugs, on average, cost 5.5 times more than those for non-orphan indications.[14]

Effective Cost Management

Managing the impact of drugs launching at higher and higher prices and concurrently wrestling with year-over-year price inflation is not simple at the best of times, but the emerging pipeline trends present an additional set of challenges. Meeting these challenges will require a deep understanding of plan member needs and clinical considerations, a method for staying abreast of changes in the evolving specialty marketplace, and an unwavering commitment to both quality patient care and proactive cost management.

Real-Time Monitoring and Dynamic Management

In a rapidly changing marketplace, opportunities to improve outcomes, boost value, and control costs emerge quickly. CVS Health actively monitors the pipeline for payors. Our interactive trend dashboard identifies drivers of cost and utilization and helps model the adoption and cost impact of new drugs. We also share Budget Impact Models with clients for drugs that have the greatest potential to impact trend. And for complex fields like oncology, our NovoLogix platform enables prior authorization across benefits while integrating current guidelines for regimen-level review. This not only provides effective cost management but is also a precision medicine tool, helping ensure that all members have the opportunity to receive the most current standard of care.

Value-Based Management

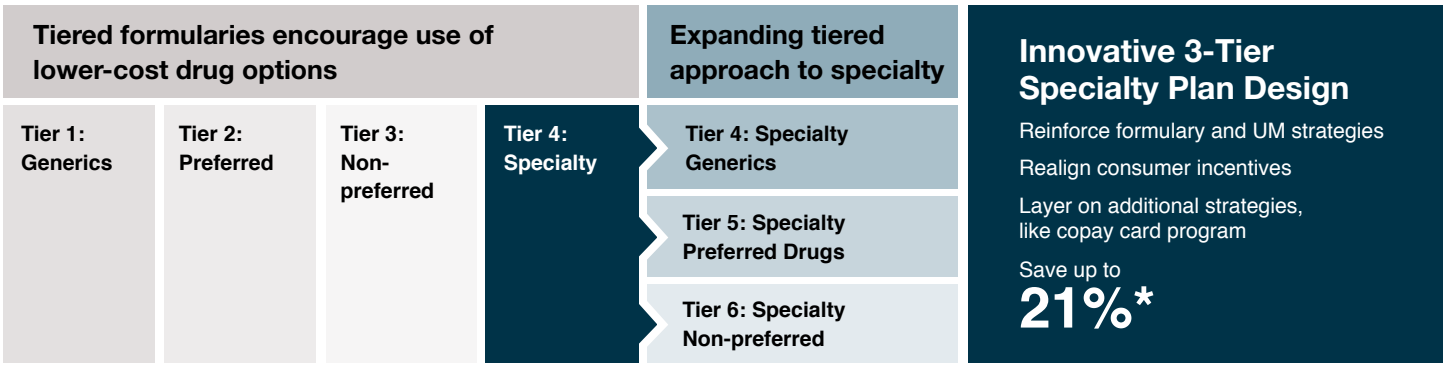
With more manufacturers seeking supplemental indications for high-cost drugs and growing competitiveness in key therapy areas, targeted management strategies such as indication- and outcome-based contracting and indication-based pricing can help payors effectively manage cost. Such strategies involve negotiating prices and rebates for a specific diagnosis rather than at a therapy class level, and tying reimbursement for a drug to the value it delivers in treating a specific condition, as measured by patient outcomes. We currently have indication-based formularies and outcomes-based contracts in place for autoimmune conditions such as rheumatoid arthritis and psoriasis, and continue to look for additional value-based management opportunities for high-cost therapy classes.

We are also innovating to help address the issue of new drug launch price escalation. In this work, we are utilizing analyses from the Institute for Clinical and Economic Review (ICER) and an advisory board of independent health economic experts to develop innovative plan designs that more closely align drug price to value.

Maximizing the Potential of Biosimilars

Biosimilars are more complex to manage than generics, but our experience is that with appropriate plan design, formulary management, and utilization management — and attention to member incentives — it is possible to get prescribers and members to take advantage of the cost benefits biosimilars offer.

Expanding Tiered Formularies to Specialty Medications



UM (Utilization management).

* CVS Specialty internal data, March 2018. Savings will vary based upon a variety of factors including things such as plan design, demographics and programs implemented by the plan.

New Modalities, New Coverage Models

Emerging categories such as gene therapies are potentially transformative, but will require similarly innovative strategies for how they are covered. Some of the most basic issues — for instance, whether emerging gene therapies should fall under the medical or pharmacy benefit — still need to be settled. The tradeoff of short-term and long-term costs, as well as mortality rates and quality of life impact over time need to be better assessed. And new financing mechanisms for addressing the high cost of therapy — such as annuity models or reinsurance — must be developed. We are actively involved in each of these crucial areas of model development.[15]

Today's drug pipeline gives us a glimpse of a new paradigm in pharmacologic therapy, where more diverse and powerful drugs will play a critical role in the treatment of both rare and common diseases, improving upon their predecessors.

Managing costs effectively and ensuring appropriate access in this rapidly evolving environment will require an understanding of the full range of therapeutic options and how they are best used, as well as the proactive implementation of solutions that help maximize the value derived from each therapy and drive toward our ultimate shared goal: better outcomes for patients.

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* New drug count includes new molecular entities, new biologics, biosimilars, new combinations, new formulations. Projections by Pipeline Services, 2018.

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