



OptumRx Drug Pipeline Insights Report

Q1 2020



Looking Ahead: 2020 Pipeline

From Sumit Dutta, Chief Medical Officer at OptumRx

At OptumRx, our responsibility is to help ensure members have access to appropriate and cost-effective medications that improve health outcomes and lower overall medical costs.

Following the theme of our previous Drug Pipeline Insights Reports, we look at several drugs expected to be approved by the FDA in the months ahead. We identified these drugs as significant because of the unique way they work and the high costs they will likely bring to the health care system. In addition, we reviewed a few critical conditions to watch this year.

This OptumRx publication is designed to increase your understanding of the pharmaceutical pipeline for 2020.

Sumit Dutta MD



SUMIT DUTTA
CHIEF MEDICAL OFFICER AT OPTUMRX

Three drugs nearing their anticipated approval date in the first half of 2020 that will have the biggest impact on payers and patients:



A New High-Cost Class of Drugs for High Cholesterol

More than 37% of Americans have elevated low-density lipoprotein-cholesterol (LDL-C), more commonly known as high cholesterol. It is well known that lowering cholesterol can protect against heart disease, heart attacks and stroke. Additionally, 18 million individuals with atherosclerotic cardiovascular disease have elevated LDL-C levels despite treatment with currently available therapies, highlighting the need for new approaches when the condition is unmanaged.

Bempedoic acid will be the first in a new class of drugs for the treatment of high cholesterol, if approved. This new drug works differently by inhibiting an enzyme [ATP-citrate lyase] involved in cholesterol production. Bempedoic acid can be taken in combination with other lipid-lowering therapies for patients who may not be at their goal with existing therapy. In clinical trials, bempedoic acid lowered LDL-C by approximately 15% to 30% compared to placebo. By itself, bempedoic acid provides similar reductions in LDL to other generically available therapies.

Since bempedoic acid is taken by mouth, we believe it will be favored over injectables like PCSK9 inhibitors, a biologic drug that significantly lowers LDL-C levels. However, many drugs are available for the management of high cholesterol, including oral generic statins. Due to this, and the long track record of available therapies, bempedoic acid is likely to be used as a second- or third-line option in patients who have failed prior treatments. Reports indicate that manufacturer pricing for bempedoic acid will be \$9 or \$10 per day, or less than \$4,000 per year.



First-of-its-Kind Treatment for Peanut Allergy

As the number of patients with food allergies grows, peanut allergy remains the most common food allergy in the U.S. It is estimated between 6 and 15 million children experience allergic symptoms when exposed to peanuts. Current care for peanut allergy is to strictly avoid peanuts and in cases of accidental exposure, use epinephrine injections. Because of the large patient population, and minimal treatment options, there is a \$4.5 billion global market for drug manufacturers to treat peanut allergies.

AR-101, if approved, would be the first oral immunotherapy to reduce allergic reactions from exposure to peanuts. AR-101 is ingested to gradually increase doses of purified peanut protein, in order to help the immune system learn to tolerate peanuts. This practice of oral immunotherapy is being offered today by some allergists but has yet been available as an FDA-approved drug.

In one clinical trial, approximately two thirds of patients taking AR-101 were able to ingest a dose of 600 mg (approximately two peanut kernels) or more of peanut protein without symptoms (e.g., moderate to severe allergic reactions), vs. 4% of patients treated with the placebo. AR-101 does have limitations, including an approximately 6 month dose escalation period where the AR-101 dose is gradually increased every 2 weeks under the care of a health care professional in order to ensure safety and tolerability. AR-101 was also associated with a higher discontinuation rate due to adverse events (e.g., nausea, abdominal pain) vs. placebo (11.6% vs. 2.4%) and a higher rate of epinephrine use during the course of the trial. However, the results of the pivotal trial demonstrate that AR-101 could substantially change the outlook for many patients who have a peanut allergy by potentially reducing the risk of severe allergic reactions from accidental peanut exposure.

Although exact pricing is not yet known, AR-101 is likely to be costly. The drug manufacturer predicted AR101 would cost \$6,000 to \$8,000 for the six-month dosing-up phase and \$4,000 to \$5,000 for a year of maintenance.

Three drugs nearing their anticipated approval date in the first half of 2020 that will have the biggest impact on payers and patients (cont.):



A Rare Disease Drug Targeting More Common Condition (Fatty Liver Disease)

Nonalcoholic steatohepatitis (NASH) is a liver condition that can lead to cancer and liver failure, affecting 16 million American adults. Because this condition has direct ties to obesity, NASH prevalence is rising and is an increasing contributor to growing liver-related morbidity and mortality statistics. At its current rate of growth, NASH is projected to become the leading indication for liver transplantation in the U.S. in 2020. Current care for the treatment of NASH consists of lifestyle modifications, including weight loss; however, many patients are unable to achieve adequate weight loss, creating a market opportunity for drug manufacturers.

If approved, obeticholic acid would be the first treatment for NASH and will likely be an attractive option for patients struggling to lose weight. In the pivotal trial for NASH, 23% of patients achieved an improvement in fibrosis (liver scarring) with no worsening of NASH with obeticholic acid (25 mg) compared to just 12% without. However, the proportion of patients experiencing resolution of NASH was no different than placebo.

Obeticholic acid is on the market already, available under the brand name Ocaliva for a rare liver disease called primary biliary cirrhosis, but it has not yet been approved for the treatment of NASH. Ocaliva is priced at around \$85,000 per patient per year. The drug manufacturer is expected to launch a new formulation of obeticholic acid under a different brand name and with indication-specific pricing. Given the large (and growing) target patient population in conjunction with the high unmet treatment need, it is projected that pricing for obeticholic acid for NASH will be between \$10,000 to \$18,000 per patient per year. It will also likely have a year or more marketing head start over related drugs, but with more than 50 drugs currently in the pipeline for treatment of NASH, this could quickly become a competitive marketplace, assuming other products achieve positive outcomes in phase 3 trials.

Top Drug Classes for 2020

OptumRx's Pipeline Drug Surveillance team monitors over 1,000 new drugs and indications at any given time.

LOOKING AHEAD TO 2020:



150 NEW DRUG APPROVALS

Over 150 new drug approvals, including new molecular entities and other approvals, will be evaluated by the FDA.



64 DRUGS FILED WITH FDA

Currently, 64 total drugs have been filed with the FDA and have anticipated approval dates in 2020.

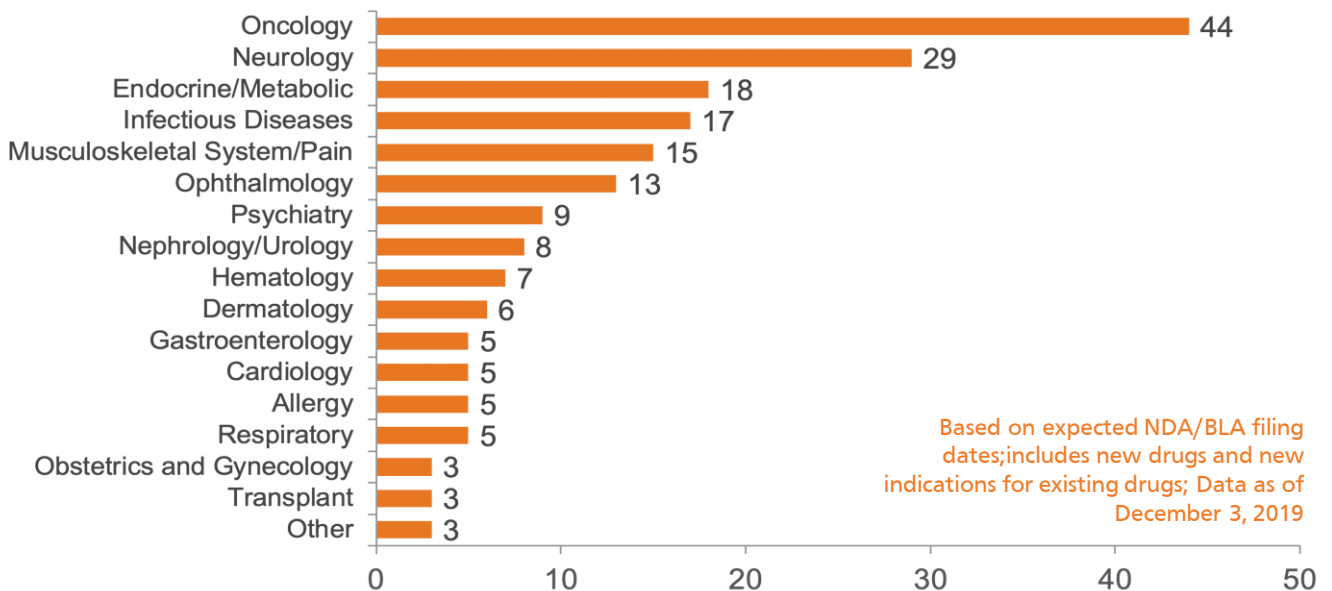


11 U.S. BLOCKBUSTERS

11 are potential blockbusters with expected \$1B+ in U.S. sales.

DRUG APPROVAL PIPELINE SNAPSHOT:

OVER 150 DRUGS* COULD BE APPROVED BY FDA IN 2020



OptumRx identified the top therapeutic disease areas and drug categories that are driving the greatest number of potential new drug entrants this year. Below are a few takeaways:

1

Oncology leads the pack

A continuation from past years, oncology is a top therapy class indicating that pharmaceutical manufacturers continue to invest heavily in this space. Within oncology, roughly 70% of anticipated approvals in 2020 will be high cost orphan drugs, with new drug developments primarily focused on very narrow populations with rare subsets of cancer defined by a biomarker or genetic mutation. In these populations, trial sizes are often small and use surrogate endpoints, making it challenging to assess the overall value of the drug. Although the impact of such drugs has historically been small, the continued growth in the number of orphan indications and products could lead to substantial growth in the overall cost of care.

In 2020 and beyond, we expect oncology to remain the drug class leader, given oncology encompasses an extremely complex set of more than 200 disease variations, each requiring more advanced research leading to different nuances in treatment.

Oncology is also one of the costliest drug classes where pharmaceutical companies have been successful in setting high prices for new drugs. Looking ahead, specialty pharmacy care management will play a critical role in ensuring these drugs are being used appropriately, reducing waste, improving safety, and improving health outcomes for these patients.

2

Treatments for neurology grow in popularity

Treatments for neurological conditions are second on the list of top drug categories and the pipeline of products treat a wide array of conditions within that class. This includes new therapies for more common conditions such as migraine, Parkinson's disease, and epilepsy, to novel therapies that treat rare or ultra-rare conditions such as spinal muscular atrophy and neuromyelitis optica.

These drugs could have significant clinical and financial impact because of the size of the population affected or because of the high unmet need for treatment options. In some cases, these pipeline agents will also be entering a relatively crowded marketplace (for example, multiple sclerosis) and this added competition could provide an opportunity for lower drug prices for available treatments.

3

Orphan drugs make up nearly 40% of drugs across disease categories – and continue to remain a priority

In the past 10 years, the number of orphan drug indications has risen dramatically. Orphan drugs – which treat conditions that affect less than 200,000 people in the U.S. – are critical for patients with rare conditions where treatments may not currently exist. In 2017, the FDA approved 80 new orphan indications, up from 40 in 2016 and just 15 in 2010. In 2018, for the first time ever, the FDA approved more new molecular entities with orphan drug designation than non-orphan drugs.

In 2020, it is expected this emphasis on orphan drugs will continue, while the number of orphan drugs proportionate to all drugs approved is expected to remain the same (with orphan drugs representing an average of 44% of all drugs approved between 2016 and 2019). Looking across all categories, oncology has the largest proportion of drugs with an orphan drug designation. Remaining orphan drugs are diverse across the categories, mostly hematology, neurology and endocrine/metabolic.

Similar to the oncology space, orphan drugs are expensive, costing on average \$147,000 a year or more, and will require utilization and specialty pharmacy care management to reduce costs for patients and clients. Given the return on investment, pharmaceutical manufacturers continue to prioritize development of orphan drugs for conditions where there is no other alternative option.

About OptumRx

OptumRx is a pharmacy care services company helping clients and more than 56 million members achieve better health outcomes and lower overall costs through innovative prescription drug benefit services, including network claims processing, clinical programs, formulary management, specialty pharmacy care and infusion services. Through expertise, flexible technology and a network of over 67,000 community pharmacies and state-of-the-art home delivery pharmacies, OptumRx is putting patients at the center of the pharmacy experience and making health care more connected and less fragmented — ensuring patients get the right medication at the right time at the best cost. OptumRx is part of Optum®, a leading information and technology-enabled health services business dedicated to making the health system work better for everyone. For more information, visit optum.com/optumrx or follow @OptumRx on Twitter.



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