Pharmacy Focus: Pipeline Therapies to Watch through 2022

What's in the Pipeline?

2022 is expected to be an exciting year for pipeline therapies finally making their way to market approval. After facing delays and setbacks, many therapies and other high-cost products are ready for Food and Drug Administration (FDA) review.[1]

Several innovative therapies in the FDA pipeline include new treatment options for hemophilia A and hemophilia B. These blood disorders are rare diseases that are commonly associated with high-cost claims based on the severity of the disease state and the type of treatment needed.[2] To date, there are no FDA approved gene therapies for either of these bleeding disorders.[3] However, they have been widely anticipated, and we expect to see activity around market entry by 2022.[4]

Roctavian™ is one of the hemophilia A products that has been vying for FDA approval for the past few years. However, delays in the approval process have prevented the product from being authorized for market use.[5]

Some reasons for delays around pipeline therapy approvals are as follows:

1. The pandemic stopped the importation of certain materials and substances for manufacturing
2. Travel restrictions have been in place for FDA inspectors both domestically and abroad
3. Priority was given to therapies of potential value for the management of SARS-CoV-2
4. The FDA required higher study populations (more participants)
5. New policies requiring a longer duration for observing the outcomes of gene therapies also were enacted[1]

These issues created delays for more than a year, and now, all signs point to 2022 being full of completed applications by manufacturers and prompt review by the FDA with price points to be considered. Many gene and cell therapies in the pipeline have previously received an expedited review designation, which ultimately means they could be approved within six to 10 months after the completed application is submitted for FDA review.[6,7]

Pipeline Gene and Cell Therapies

Disease	Drug*	Approval Status
AADC Deficiency	Eladocagene exuparvovec	Review date: As early as 2022[8]
Achondroplasia	Voxzogo™ (vasoritide)	Review date: 11/21[9]
Beta Thalassemia	Zynteglo® (betibeglogene autotemcel)	Review date: Q2 2022[7]
Cerebral Adrenoleukodystrophy	Skysona™ (elivaldogene autotemcel)	Review date in 2022[7]
Choroideremia	Timrepigene emparvovec	Review date: As early as 2022[10]
Duchenne Muscular Dystrophy	Translarna™ (ataluren)	Review date: As early as 2022[7]
Multiple Myeloma	Cilta-Cel (ciltacabtagene autoleucel) Abecma® (idecabtagene vicleucel)	Review date: 11/21 Possible approval for expanded use in 2022[11]
Epidermolysis Bullosa	B-Vec (beremagene geperpavec) EB-101	Review date: As early as 2022[7]

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Pipeline Gene and Cell Therapies, *continued*

Disease	Drug*	Approval Status
Hemophilia A	Roctavian™ (valoctocogene roxaparvovec) Giroctocogene fitelparvovec	Review date: Q4 2022[7]
Hemophilia B	Etranacogene dexaparvovec Fidanacogene elaparvovec	Review date: 12/22[7]
Lebar's Hereditary Optic Neuropathy	Lumevoq® (lenadogene nolparvovec)	Review date: As early as 2022[7]
Mantle Cell Lymphoma (a subtype of NHL)	Tecartus™ (brexucabtagene autoleucel)	Approved for expanded use 10/01/21[7]
Marginal Zone Lymphoma (a subtype of NHL)	Yescarta® (axicabtegene ciloleucel)	Review date: Q2 2022[12]
Non-muscle Invasive Bladder Cancer	Adstiladrin® (nadofaragene firadevovec)	Review date: Q2 2022[7]
Refractory Angina due to Myocardial Ischemia	Generx™ (alferminogene tadenovec)	Review date: 6/22[7]
Sickle Cell Disease	LentiGlobin™ (betibeglogene autotomcel)	Review date in 2022[13]
Spinal Muscular Atrophy	Zolgensma® (onasemnogene abeparvovec)	Review date for expanded use in older patients: 12/22[14]

**If common names for therapies are listed, such names are not guaranteed to remain the same upon market approval.*

Other Pipeline Therapies[7]

Disease	Therapies*
Alport Syndrome	1
Alzheimer's Disease	2
Arginase 1 Deficiency	1
Cerebral adrenoleukodystrophy	1
Cushing Syndrome	2
Hemophilia A	1
HIV	1
Myasthenia Gravis	1
Myelodysplastic Syndrome	4

Disease	Therapies*
Neimann-Pick Disease	2
NSCLC	1
Ovarian Cancer	1
PHN	2
Pompe Disease	1
Pyruvate Kinase Deficiency	2
Rett Syndrome	1
Tay-Sachs	1
Wilson's Disease	1

All therapies are in late phases of clinical trials.
**Indicates number of possible drug/therapies for listed disease state that could receive market approval in 2022.*

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Sickle Cell & Beta Thalassemia

Sickle cell disease and beta thalassemia are two somewhat similar diseases for which gene therapies are now in the final phases of clinical trials in the United States. Even though these are two distinct diseases, they share some similarities in treatment methods. Both sickle cell and beta thalassemia can be treated with blood transfusions and other therapies that help to manage the complications that are commonly present in both disease states.[1,4]

There are already established standards in care, one of which is a hemopoietic stem cell transplant (HSCT). HSCT is the only therapy that is considered a possible curative treatment, as opposed to traditional symptomatic management.[3, 16, 17] However, even with the improving disease-free survival rates from HSCT, the therapy is not without significant risks, including transplant rejection, infection risks and other complications.[17, 18]

The new gene therapies that are in the pipeline are being assessed for their ability to assist patients who are not eligible for an HSCT as a one-time infusion.[18] And while this may become a treatment option in the future, it is important to remember that gene therapies have not been proven to be curative, and not all gene therapies are intended to have similar outcomes. For example, Zolgensma® was created to slow down disease progression and hopefully expand the time of independence for infants with the genetic defect causing spinal muscular atrophy, while the pipeline gene therapies for blood disorders like hemophilia are showing an opportunity to increase convenience, at least temporarily, by reducing the number (or volume) of required treatment administrations for some amount of time.[19]

The globe is keeping close watch on the data around the new treatments being developed for both sickle cell disease and beta thalassemia, in which significant outcomes from their trials are still being anticipated.[20] Branded as Zynteglo™, the beta thalassemia gene therapy has already been withdrawn from the German market for the inability to contract cost vs. quality and is already losing momentum after its recent approval in Europe.[20]

Cost Containment Considerations

As part of its HMConnects™ cost containment program, HM Insurance Group works to support cost management opportunities around the use of gene and cell therapies. The Pharmacy Operations (RxOps) team not only watches the pipeline, but also anticipates how it will impact HM's client base with regard to current or potential risks. Reviewing, auditing and collaborating on the content of current policies, monitoring trends and implementing appropriate cost savings techniques are standard practices that are provided to our clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the self-funded insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com**.

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