

2025 Revenue

\$12.0 billion

2025 EBITDA (Est.)

\$6.05 billion

2025 Net Income

\$4.7 billion

Vertex Pharmaceuticals: Deep Dive Research Report

March 2026

Executive Summary

Company Overview: Vertex Pharmaceuticals is a global biotechnology company headquartered in Boston, Massachusetts. Founded in 1989, the company is a leader in the treatment of cystic fibrosis (CF) and is expanding into other serious diseases. As of the latest reports, Vertex employs over 5,000 professionals worldwide.

Key Financial Metrics (Fiscal Year 2025): * **Revenue:** \$12.0 billion * **EBITDA (Estimated):** \$6.05 billion * **EBITDA Margin (Estimated):** 50.4% * **Net Income:** \$4.7 billion

Strategic Position and Growth Trajectory: Vertex maintains a dominant position in the cystic fibrosis market with its portfolio of transformative medicines. The company's growth strategy is focused on expanding its leadership in CF, advancing a broad and deep pipeline in other serious diseases, and leveraging its strong financial position for strategic investments.

Forward-Looking Disclaimer: This report contains forward-looking statements based on current expectations and available data as of March 2026. Actual results may differ materially due to risks outlined in the Risks section.

1. Company History

Founding and Early Years (1989-1990s): Vertex Pharmaceuticals was founded in 1989 by Dr. Joshua Boger and Kevin J. Kinsella in Cambridge, Massachusetts. The company pioneered a strategy of rational drug design, a departure from the more common combinatorial chemistry approach. The company went public in 1991 with an IPO on NASDAQ. The early years were focused on antiviral research, leading to collaborations with GlaxoSmithKline and the launch of their first product, Agenerase (an HIV protease inhibitor), in 1999.

Growth and Expansion (2000s): The 2000s saw Vertex expand its research focus and capabilities. A key move was the acquisition of Aurora Biosciences in 2001, which enhanced the company's drug discovery and screening capabilities. During this period, Vertex's pipeline grew to include programs in viral infections, inflammation, autoimmune disorders, and cancer.

The Cystic Fibrosis Breakthrough (2010s): The 2010s marked a transformative period for Vertex, as the company shifted its focus to cystic fibrosis (CF). This resulted in the development and launch of a series of groundbreaking medicines that treat the underlying cause of CF. * **2012:** Kalydeco (ivacaftor) is approved by the FDA, the first medicine to treat the underlying cause of CF in people with specific mutations. * **2015:** Orkambi (lumacaftor/ivacaftor) is approved, expanding treatment to a larger group of CF patients. * **2018:** Symdeko (tezacaftor/ivacaftor and ivacaftor) is approved. * **2019:** Trikafta (elexacaftor/tezacaftor/ivacaftor and ivacaftor) is approved, a triple-combination therapy that significantly expanded the number of CF patients eligible for treatment.

Recent Developments and Future Directions (2020s): In the 2020s, Vertex has continued to strengthen its leadership in CF while also aggressively expanding its pipeline into other serious diseases. The company has made several strategic acquisitions, including Semma Therapeutics (2019) for type 1 diabetes, Exonics Therapeutics (2019) for Duchenne muscular dystrophy, and ViaCyte (2022) to further bolster its cell therapy platform. In 2024, Vertex announced the acquisition of Alpine Immune Sciences for \$4.9 billion, a move that significantly enhances its immunology pipeline.

Leadership Transitions: * **Dr. Joshua Boger (1989-2009):** Co-founder and first CEO of Vertex, Dr. Boger led the company for its first 20 years. * **Dr. Jeffrey Leiden (2009-2020):** Dr. Leiden succeeded Dr. Boger and oversaw the company's transformation into the dominant player in the CF market. He transitioned to the role of Executive Chairman in 2020. * **Dr. Reshma Kewalramani (2020-Present):** Dr. Kewalramani became CEO in 2020, having previously served as the company's Chief Medical Officer. She is leading Vertex's next phase of growth and pipeline diversification.

2. Ownership Structure

Public/Private Status: Vertex Pharmaceuticals is a publicly-traded company, listed on the NASDAQ stock exchange under the ticker symbol VRTX.

Major Shareholders: The company is predominantly owned by institutional investors. While the exact percentages fluctuate with each filing period, the largest and most consistent shareholders include:

- **Capital World Investors:** ~10-11%
- **The Vanguard Group:** ~9-9.5%
- **BlackRock:** ~5.7-9.4%
- **Capital Research Global Investors:** ~6.3-6.5%
- **State Street:** ~4.6%

Data Gap Note: The ownership percentages are based on the latest available 13F filings and may not reflect the most current ownership structure.

Board of Directors: The Board of Directors is composed of 10 members with a diverse range of experience across science, medicine, and business. The board as of the latest available information is comprised of:

- Dr. Jeffrey Leiden (Chairman)
- Dr. Reshma Kewalramani (CEO)
- Bruce Sachs
- Dr. Sangeeta Bhatia
- Dr. Alan Garber
- Lloyd Carney
- Nia Tatsis
- Diana McKenzie
- Suketu Upadhyay
- Michel Lagarde

3. Leadership / C-Suite

Dr. Reshma Kewalramani - Chief Executive Officer and President: Dr. Kewalramani has served as CEO and President of Vertex since April 2020. She joined the company in 2017 as Chief Medical Officer and Executive Vice President of Global Medicines Development and Medical Affairs. Under her leadership, Vertex has expanded its leadership in cystic fibrosis, brought new therapies to market, and significantly grown its pipeline. Prior to Vertex, Dr. Kewalramani spent

over 12 years at Amgen in various leadership roles. She is a trained physician, specializing in internal medicine and nephrology.

Charles F. Wagner - Executive Vice President, Chief Financial Officer and Chief Operating Officer: Mr. Wagner has been the CFO of Vertex since April 2019 and recently added the role of COO. He is responsible for the company's financial and operational functions. Before joining Vertex, Mr. Wagner was the CFO at Ortho Clinical Diagnostics and Bruker Corporation. He holds an MBA from Harvard Business School.

Dr. Carmen Bozic - Executive Vice President, Global Medicines Development and Medical Affairs and Chief Medical Officer: Dr. Bozic joined Vertex in 2019 and was appointed CMO in 2020. She leads the clinical development and medical affairs teams. Prior to Vertex, Dr. Bozic had a long career at Biogen, where she held senior leadership roles in global development. She is a physician, trained in internal medicine and pulmonary/critical care medicine.

Ludovic Fenaux - Senior Vice President, International Commercial Operations: Mr. Fenaux has been with Vertex since 2018 and leads the company's commercial operations outside of the United States. He has extensive experience in the biopharmaceutical industry, having spent 15 years at Bristol Myers Squibb in various senior commercial roles.

4. Drug Portfolio

Cystic Fibrosis

Product	Revenue (2025 Est.)	Growth	Patent/Exclusivity Status (US)	Peak Sales Potential (Franchise)	P&L Owner
Trikafta/Kaftrio	~\$9.83 billion	Strong Growth	2037	\$12.5 - \$13.5 billion	Not publicly confirmed
Symdeko/Symkevi	~\$0.80 billion	Declining	Not specified	"	"
Orkambi	~\$0.50 billion	Declining	2026	"	"

Product	Revenue (2025 Est.)	Growth	Patent/Exclusivity Status (US)	Peak Sales Potential (Franchise)	P&L Owner
Kalydeco	~\$0.37 billion	Declining	2026 (new formulation)	"	"

Revenue figures are estimated based on public disclosures of total CF franchise revenue and market share data.

Detailed Write-ups:

Trikafta/Kaftrio (elexacaftor/tezacaftor/ivacaftor and ivacaftor): Trikafta is a combination of three drugs that target the defective CFTR protein. It is considered a breakthrough therapy and is approved for a broad range of CF patients, representing about 90% of the CF population. The drug has been the primary driver of Vertex's revenue growth since its launch in 2019. Its long patent life provides a durable revenue stream for the company.

Symdeko/Symkevi (tezacaftor/ivacaftor and ivacaftor): Symdeko is a combination therapy that was approved prior to Trikafta. While it has been a successful product, its use is declining as patients are switched to the more effective Trikafta.

Orkambi (lumacaftor/ivacaftor): Orkambi was the first combination therapy to treat the underlying cause of CF for patients with two copies of the F508del mutation. Like Symdeko, its use is declining with the adoption of Trikafta.

Kalydeco (ivacaftor): Kalydeco was Vertex's first CFTR modulator and a groundbreaking medicine that treats the underlying cause of CF in patients with specific mutations. While its use is limited to a smaller subset of patients, it remains an important therapy.

Other Therapeutic Areas

Product	Revenue (2025)	Growth	Patent/Exclusivity Status (US)	Peak Sales Potential	P&L Owner
Casgevy	\$116 million	N/A (Launch Year)	Foundational patents to 2034	Not Available	Not publicly confirmed

Product	Revenue (2025)	Growth	Patent/Exclusivity Status (US)	Peak Sales Potential	P&L Owner
Journavx	\$60 million	N/A (Launch Year)	2040	~\$4.9 billion	"
Zimislecel	N/A (Pre-launch)	N/A	Not Available	Not Available	"
Inaxaplin	N/A (Pre-launch)	N/A	Not Available	Not Available	"
Povetacicept	N/A (Pre-launch)	N/A	Not Available	Not Available	"

Detailed Write-ups:

Casgevvy (exagamglogene autotemcel): Casgevvy is a groundbreaking gene therapy for sickle cell disease and beta thalassemia, developed in partnership with CRISPR Therapeutics. It is one of the first approved therapies based on CRISPR gene-editing technology. While the initial launch has been slow due to the complexity and cost of treatment, it represents a significant step into a new therapeutic area for Vertex.

Journavx (suzetrigine): Journavx is a first-in-class, non-opioid pain medication for the treatment of moderate-to-severe acute pain. The launch of Journavx is a key part of Vertex's strategy to diversify beyond cystic fibrosis. The drug has significant market potential, given the high unmet need for effective, non-addictive pain treatments.

Zimislecel (formerly VX-880): Zimislecel is an investigational cell therapy for type 1 diabetes. The therapy involves transplanting stem cell-derived islet cells to replace the insulin-producing cells that are destroyed in type 1 diabetes. Early clinical data has been promising, and Zimislecel has the potential to be a functional cure for the disease.

Inaxaplin (VX-147): Inaxaplin is an investigational small molecule inhibitor of APOL1 for the treatment of APOL1-mediated kidney disease, a rare and serious kidney disease. The drug has received orphan drug designation and is currently in late-stage clinical trials.

Povetacicept (ALPN-303): Povetacicept is an investigational dual BAFF/APRIL inhibitor for the treatment of IgA nephropathy and other autoimmune diseases. The drug was acquired through

the acquisition of Alpine Immune Sciences and is a key part of Vertex's strategy to build a leading immunology franchise.

5. Top 5 CEO Focus Drugs

Based on recent earnings calls and investor presentations, the following five drugs represent the key strategic priorities for Vertex's leadership team:

- 1. Trikafta/Kaftrio:** As the cornerstone of the multi-billion dollar cystic fibrosis franchise, Trikafta remains a top priority. The focus is on maximizing its reach to all eligible patients globally, expanding its label to younger age groups, and defending its market position.
 - 2. Casgevy:** Casgevy represents Vertex's bold entry into gene therapy. The successful launch and commercialization of this one-time treatment for sickle cell disease and beta thalassemia is a critical step in the company's diversification strategy.
 - 3. Journavx:** The launch of Journavx, a first-in-class non-opioid pain medication, is another key pillar of the diversification strategy. The company is focused on driving adoption and establishing Journavx as a leading treatment for acute pain.
 - 4. Zimislecel:** This investigational cell therapy for type 1 diabetes is a high-risk, high-reward program that has the potential to be a functional cure for the disease. The CEO has highlighted the transformative potential of Zimislecel, and the company is investing heavily in its development.
 - 5. Povetacicept:** Acquired through the Alpine Immune Sciences acquisition, povetacicept is a late-stage asset for IgA nephropathy and other autoimmune diseases. It is the lead asset in Vertex's emerging immunology pipeline and has the potential to be a "pipeline-in-a-product" with multiple indications.
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6. P&L Owners

The following are the key therapeutic areas for Vertex and the status of identifying the respective P&L owners or franchise heads. This information is not typically disclosed publicly.

- **Cystic Fibrosis:** Not publicly confirmed
- **Hematology (Sickle Cell Disease & Beta Thalassemia):** Not publicly confirmed
- **Pain:** Not publicly confirmed
- **Type 1 Diabetes:** Not publicly confirmed
- **Immunology:** Not publicly confirmed

C-Suite LinkedIn Profiles: * Reshma Kewalramani (CEO & President):

<https://www.linkedin.com/in/reshma-kewalramani-md-vertex/> * **Charles F. Wagner (EVP, CFO & COO):** Not publicly confirmed * **Carmen Bozic (EVP & CMO):** Not publicly confirmed
 * **Ludovic Fenaux (SVP, International Commercial):** Not publicly confirmed

7. R&D Pipeline

Program	Indication	Phase	Expected Readouts/Milestones (2026)	Partnerships
Zimislecel	Type 1 Diabetes	Phase 3	Approval submission planned	N/A
Inaxaplin	APOL1-mediated Kidney Disease	Phase 2/3	Interim analysis, potential for accelerated approval filing	N/A
Povetacicept	IgA Nephropathy	Phase 3	Interim analysis, potential for accelerated approval filing	N/A
Povetacicept	Generalized Myasthenia Gravis	Phase 2	Study initiation	N/A
Vanazalanib	Autosomal Dominant Polycystic Kidney Disease (ADPKD)	Phase 3	Ongoing	N/A
VX-522	AAT Deficiency	Phase 2	Ongoing	Moderna (mRNA)
VX-984	Pain	Phase 2	Ongoing	N/A

8. Top Geographies

Region	Revenue (2025 Est.)
United States	~\$7.55 billion
International	~\$4.45 billion
Total	~\$12.0 billion

Revenue figures are estimated based on 2024 reported geographic revenue and 2025 growth rates.

9. Competitive Landscape

Cystic Fibrosis: Vertex enjoys a near-monopoly in the cystic fibrosis market, with a market share exceeding 90%. The company's portfolio of CFTR modulators has transformed the treatment of the disease and has no direct competitors for the majority of patients. While some smaller biotech companies are developing therapies for the ~10% of patients who do not respond to Vertex's drugs, the company's dominant position is not expected to be challenged in the foreseeable future.

Sickle Cell Disease: In the emerging field of gene therapy for sickle cell disease, Vertex's Casgevy faces its primary competition from Bluebird Bio's Lyfgenia. Both therapies were approved around the same time and are priced similarly. The market is still in its early stages, and it is unclear which therapy will ultimately gain a larger market share.

Acute Pain: Journavx competes in the crowded and well-established acute pain market. Its main competitors are generic opioids, which are inexpensive and effective, but have significant safety and addiction concerns. Journavx's non-opioid mechanism of action is its key differentiating feature, but its higher price point and perceived lower efficacy compared to opioids may be barriers to adoption.

Type 1 Diabetes: The type 1 diabetes market is currently dominated by insulin therapies. Vertex's Zimislecel, if successful, would be a disruptive, potentially curative therapy. Its main competitors are other companies developing cell-based therapies, such as Sana Biotechnology. The only currently approved cell therapy is Lantidra, which is based on donor-derived islets and has limited availability.

IgA Nephropathy: The IgA nephropathy market is becoming increasingly competitive. Povetacept's main competitors include Otsuka's recently approved Voyxact and Vera

Therapeutics' atacicept, which is in late-stage development. Several other large and small pharmaceutical companies are also developing therapies for this disease.

10. Risks and Challenges

Competition: While Vertex faces limited direct competition in its core cystic fibrosis franchise, the company is entering more competitive markets as it diversifies. The sickle cell disease, pain, and immunology markets all have established players and other companies with late-stage pipelines.

Patent Cliffs: Vertex is not facing a major patent cliff in the near-term, as the key patents for its blockbuster CF drug, Trikafta, do not expire until 2037. However, the patents for its older CF drugs are expiring, which will lead to generic competition for those products.

Regulatory/Pricing Pressure: As a company with high-priced drugs for rare diseases, Vertex is under constant scrutiny from payers and governments. The company faces ongoing pricing and reimbursement negotiations in many countries, and the Inflation Reduction Act in the United States could have a significant impact on its future revenues.

FX Exposure: With a significant and growing portion of its revenue coming from outside the United States, Vertex is exposed to foreign exchange rate fluctuations. A strengthening of the US dollar could negatively impact the company's reported revenues.

Pipeline Execution Risk: Vertex's long-term growth is highly dependent on the successful execution of its R&D pipeline. The company has a number of high-risk, high-reward programs in its pipeline, and any clinical trial failures or regulatory setbacks could have a significant negative impact on the company's stock price and future growth prospects.

11. Outlook and Valuation Context

Medium-Term Targets: Vertex has not provided explicit medium-term financial targets, but the company's 2026 revenue guidance of \$12.95 - \$13.1 billion implies a continued focus on strong growth from its CF franchise and the successful commercialization of its new products.

Valuation: As of March 2026, Vertex has a market capitalization of approximately \$120 billion and an enterprise value of approximately \$113 billion.

Peer Comparison Table:

Company	Market Cap	Enterprise Value	Revenue (TTM)	P/E Ratio (TTM)
Vertex Pharmaceuticals	~\$120 billion	~\$113 billion	\$12.0 billion	~25x (Est.)
Regeneron Pharmaceuticals	~\$81 billion	~\$72 billion	\$14.3 billion	~18.5x
CRISPR Therapeutics	~\$5.2 billion	~\$3.1 billion	\$37 million	N/A
Bluebird Bio	~\$49 million	~\$187 million	\$104 million	N/A

Growth Narrative: Vertex's growth narrative is centered on two key pillars: 1. **Maintaining and expanding its leadership in cystic fibrosis:** The company is focused on maximizing the reach of its existing CF therapies and advancing its next-generation CFTR modulators. 2.

Diversifying into new therapeutic areas: Vertex is leveraging its scientific expertise and strong financial position to build a pipeline of transformative medicines in other serious diseases, including sickle cell disease, pain, type 1 diabetes, and immunology.

The successful execution of this strategy has the potential to drive significant long-term growth and create substantial value for shareholders.

12. Patent/Exclusivity Summary Table

Product	Indication	US Exclusivity	EU Exclusivity	Key Risk
Trikafta/Kaftrio	Cystic Fibrosis	2037	Not Available	Long-term safety, pricing pressure
Symdeko/Symkevi	Cystic Fibrosis	Not specified	Not Available	Patient switching to Trikafta
Orkambi	Cystic Fibrosis	2026	2026	Patient switching to Trikafta

Product	Indication	US Exclusivity	EU Exclusivity	Key Risk
Kalydeco	Cystic Fibrosis	2026	2026	Patient switching to Trikafta
Casgevy	Sickle Cell Disease & Beta Thalassemia	Foundational patents to 2034	Not Available	Slow adoption, complex manufacturing
Journavx	Acute Pain	2040	Not Available	Competition from generics, pricing pressure

Ada Patient Finder Analysis

Cystic Fibrosis (CF)

Approved Indications: Vertex's CFTR modulators (Trikafta, Symdeko, Orkambi, Kalydeco) are approved for the treatment of cystic fibrosis in patients with specific mutations in the CFTR gene. Trikafta is approved for the broadest population, covering approximately 90% of CF patients.

Patient Finder Analysis:

- **(a) Diagnostic Delay:** Significant diagnostic delays exist, particularly in minority populations. One study found that 11.8% of new CF diagnoses in the US from 2001-2020 were delayed by more than 180 days.
- **(b) Per-Patient Revenue >\$100K/year:** Yes. The estimated net revenue per patient for international patients is over \$120,000 per year.
- **(c) Underdiagnosis Rate >20%:** Yes. It is estimated that 35% of CF patients worldwide are undiagnosed.

Drug-Addressable Funnel:

- **Total Prevalence (Global):** ~162,000
- **Undiagnosed:** ~57,000 (35%)
- **Eligible for Vertex Drugs:** ~146,000 (90%)
- **Addressable by Ada:** Significant portion of the undiagnosed population.

Ada's Role: Ada is well-positioned to identify patients with undiagnosed cystic fibrosis. The platform's ability to collect and analyze a wide range of symptoms, including respiratory, digestive, and constitutional symptoms, can help to surface patients who may have been missed by traditional screening methods. The significant diagnostic delay and high underdiagnosis rate, particularly in non-white populations, represent a clear opportunity for Ada to improve diagnosis and connect patients to care.

Patient Finder Fit Score: 8/10

Pitch Hook: With an estimated 35% of cystic fibrosis patients globally going undiagnosed, and significant diagnostic delays even in developed countries, there is a critical need to identify these missing patients. Ada's platform can help to close this diagnostic gap by identifying individuals with symptom patterns suggestive of CF, connecting them to appropriate testing and care, and ultimately expanding the market for Vertex's life-changing therapies.

Sickle Cell Disease (SCD)

Approved Indications: Casgevy is approved for the treatment of sickle cell disease in patients 12 years and older with recurrent vaso-occlusive crises (VOCs).

Patient Finder Analysis:

- **(a) Diagnostic Delay:** While specific data on diagnostic delay is not available, the significant underestimation of mortality and prevalence suggests that delays in diagnosis are a major issue.
- **(b) Per-Patient Revenue >\$100K/year:** Yes. Casgevy is a one-time gene therapy with a list price of \$2.2 million.
- **(c) Underdiagnosis Rate >20%:** Likely. While a specific rate is not available, studies have shown that the true mortality rate from SCD is 11 times higher than recorded, indicating a massive underdiagnosis problem.

Drug-Addressable Funnel:

- **Total Prevalence (US):** ~100,000
- **Undiagnosed:** Not quantified, but likely significant.
- **Eligible for Casgevy (recurrent VOCs):** A subset of the total prevalent population.
- **Addressable by Ada:** Significant portion of the undiagnosed population, particularly those with non-specific symptoms.

Ada's Role: Ada can play a crucial role in identifying patients with undiagnosed sickle cell disease. The platform can help to connect the dots between seemingly unrelated symptoms, such as pain crises, anemia, and organ damage, which can often be missed or misdiagnosed. By identifying patients with a high probability of SCD, Ada can help to reduce the diagnostic delay and connect patients to the specialized care they need.

Patient Finder Fit Score: 7/10

Pitch Hook: The true burden of sickle cell disease is hidden by a massive underdiagnosis problem, with mortality rates 11 times higher than official statistics. Ada can help to uncover this hidden patient population by identifying individuals with the characteristic symptom patterns of SCD, enabling them to access transformative therapies like Casgevy and improving outcomes for this underserved community.

Acute Pain

Approved Indications: Journavx is approved for the treatment of moderate-to-severe acute pain in adults.

Patient Finder Analysis:

- **(a) Diagnostic Delay:** Not applicable. Acute pain is a symptom, not a disease. However, there is a significant issue of mismanagement of acute pain.
- **(b) Per-Patient Revenue >\$100K/year:** No. The estimated revenue per patient is very low, likely under \$200.
- **(c) Underdiagnosis Rate >20%:** Not applicable.

Drug-Addressable Funnel:

- **Total Prevalence (US):** Millions of patients experience acute pain each year.
- **Undiagnosed:** Not applicable.
- **Eligible for Journavx:** Patients with moderate-to-severe acute pain who are seeking non-opioid alternatives.
- **Addressable by Ada:** While Ada can help patients understand the cause of their acute pain, the low per-patient revenue makes it a poor fit for the Patient Finder program.

Ada's Role: Ada can help users to understand the potential causes of their acute pain and guide them to appropriate care. However, due to the low per-patient revenue and the nature of acute pain as a symptom, Journavx is not a good fit for the Ada Patient Finder program.

Patient Finder Fit Score: 1/10

Pitch Hook: Not applicable.

Type 1 Diabetes

Target Indication: Zimislecel is being developed for the treatment of type 1 diabetes.

Patient Finder Analysis:

- **(a) Diagnostic Delay:** There is a significant diagnostic delay in type 1 diabetes, with studies showing that up to 21% of pediatric cases are delayed. This delay can lead to serious complications, such as diabetic ketoacidosis (DKA).
- **(b) Per-Patient Revenue >\$100K/year:** Highly likely. As a potentially curative cell therapy, Zimislecel is expected to have a very high price point.
- **(c) Underdiagnosis Rate >20%:** Not clearly quantified, but the significant diagnostic delay suggests that many cases are not being identified in a timely manner.

Drug-Addressable Funnel:

- **Total Prevalence (US):** ~1.6 million
- **Undiagnosed:** Not quantified, but a significant number of patients experience delayed diagnosis.
- **Eligible for Zimislecel:** A subset of the total prevalent population, likely those with more severe or difficult-to-manage disease.
- **Addressable by Ada:** Significant portion of the population with delayed diagnosis or atypical symptoms.

Ada's Role: Ada can help to reduce the diagnostic delay in type 1 diabetes by identifying individuals with the classic symptoms of the disease, such as polyuria, polydipsia, and weight loss. The platform can also help to identify individuals with atypical presentations, who may be at higher risk for delayed diagnosis. By flagging these individuals for further testing, Ada can help to ensure that they receive a timely diagnosis and are connected to appropriate care.

Patient Finder Fit Score: 9/10

Pitch Hook: The significant delay in diagnosing type 1 diabetes can have devastating consequences. Ada can help to shorten this delay by identifying individuals at high risk for the disease, enabling them to access potentially curative therapies like Zimislecel and transforming the management of this chronic condition.

IgA Nephropathy

Target Indication: Povetacicept is being developed for the treatment of IgA nephropathy.

Patient Finder Analysis:

- **(a) Diagnostic Delay:** There is a significant diagnostic delay in IgA nephropathy, with many patients being diagnosed at an advanced stage of the disease.
- **(b) Per-Patient Revenue >\$100K/year:** Highly likely. As a novel biologic for a rare and serious disease, povetacicept is expected to have a high price point.
- **(c) Underdiagnosis Rate >20%:** Likely. While a specific rate is not available, the late-stage diagnosis of many patients suggests that a significant number of cases are missed in the early stages.

Drug-Addressable Funnel:

- **Total Prevalence (US):** ~100,000 - 150,000
- **Undiagnosed:** Not quantified, but likely a significant portion of early-stage patients.
- **Eligible for Povetacicept:** A subset of the total prevalent population, likely those with more progressive disease.
- **Addressable by Ada:** Significant portion of the population with undiagnosed or early-stage disease.

Ada's Role: Ada can help to identify patients with early-stage IgA nephropathy by flagging individuals with persistent hematuria or proteinuria, which are often the first signs of the disease. By encouraging these individuals to seek further medical evaluation, Ada can help to reduce the diagnostic delay and enable earlier intervention with therapies like povetacicept, potentially slowing the progression to kidney failure.

Patient Finder Fit Score: 8/10

Pitch Hook: IgA nephropathy is a silent disease that often goes undiagnosed until it has caused irreversible kidney damage. Ada can help to break this silence by identifying patients at high risk for the disease, enabling them to access novel therapies like povetacicept and preserving their kidney function for years to come.