

# Vertex Pharmaceuticals Patient Finder Analysis

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Ada Cockpit Intelligence Report | March 2026

## Executive Summary

This analysis evaluates nine Vertex Pharmaceuticals drugs/programs for suitability with Ada Patient Finder. The assessment considers drug-addressable undiagnosed patients, per-patient economics (WAC/net pricing after rebates), diagnostic delays, Ada's ability to surface patients via symptom assessment, and Vertex's strategic motivation.

### Key Findings:

- **Tier 1 (Highest Priority):** Trikafta/Kaftrio (CF), Zimislecel (T1D), Inaxaplin (APOL1 kidney disease)
- **Tier 2 (Strong Fit):** Casgevy (SCD/beta-thal), Povetacicept (IgA nephropathy)
- **Tier 3 (Lower Priority):** Vanazalanib (ADPKD), VX-522 (AAT deficiency)
- **NO (Poor Fit):** Journavx (acute pain), Kalydeco/Orkambi/Symdeko (older CF drugs)

**Total Ada Revenue Opportunity (Tier 1 + 2, USA only, 5% capture):**

**Low: \$161.5M annually | High: \$243.0M annually**

## Portfolio Summary

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Drug	Indication	Tier	Fit Score	Addressable Undiagnosed (USA)	Ada Revenue Opportunity (5% capture, USA)
<b>Trikafta/Kaftrio</b>	<b>Cystic Fibrosis</b>	<b>1</b>	<b>9.5/10</b>	<b>19,440</b>	<b>\$14.9M - \$22.4M/yr</b>
<b>Zimislecel</b>	<b>Type 1 Diabetes</b>	<b>1</b>	<b>9.5/10</b>	<b>7,500</b>	<b>\$30M - \$45M (one-time)</b>
<b>Inaxaplin</b>	<b>APOL1 Kidney Disease</b>	<b>1</b>	<b>8.0/10</b>	<b>250,000</b>	<b>\$120M - \$180M/yr</b>
<b>Casgevy</b>	SCD / Beta-Thal	2	7.5/10	2,925	\$19.3M - \$28.9M (over 3yr)
<b>Povetacicept</b>	IgA Nephropathy	2	8.0/10	17,500	\$7.35M - \$11.03M/yr
<b>Vanazalanib</b>	ADPKD	3	5.5/10	20,000	\$6M - \$9M/yr
<b>VX-522</b>	AAT Deficiency	3	6.5/10	79,000	\$37.9M - \$56.9M/yr
Journavx	Acute Pain	NO	1.0/10	N/A	Not viable
Kalydeco/Orkambi/Symdeko	CF (older drugs)	NO	2.0/10	N/A	Not applicable

## TIER 1: Highest Priority Assets

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# 1. TRIKAFTA/KAFTRIO (Cystic Fibrosis)

Fit Score: 9.5/10

## Market Opportunity

USA Addressable Undiagnosed: 19,440 patients

Net Revenue/Patient: \$192,000/year

Ada Fee/Patient: \$15,360 - \$23,040/year

2025 Revenue: \$9.8B

Patent Expiry: 2037 (US)

Market	Total CF Prevalence	Underdiagnosis Rate	Undiagnosed Patients	Drug-Addressable (90%)
USA	~61,600	35.1%	~21,600	19,440
DACH	~25,000-34,000	12-18%	~3,000-6,000	~4,000
ROW	~65,000	50-60%	~50,000-80,000	~58,500

Sources: Cystic Fibrosis Foundation 2024, CF News Today 2024 (35.1% global underdiagnosis), JAMA 2024, ECFS Patient Registry 2023

## Clinical Profile

**Primary Symptoms:** Chronic cough with thick mucus, wheezing, recurrent chest/sinus infections, greasy stools, poor weight gain despite normal appetite, salty-tasting skin, pancreatic insufficiency

**Diagnostic Delay:** USA median 21 days for newborns (2023), BUT significant delays in minorities—up to 4+ years in some cases. 21% of diagnoses delayed >180 days. Hispanics 24% of delayed cases vs 8-9% of CF population.

**Ada Surface Ability: 9/10** - Strong symptom constellation (respiratory + digestive + growth delays). Family history adds signal. Distinctive pattern differentiates from asthma, GERD.

Sources: Lurie Children's Hospital CF Report 2024, PMC 2024 (racial disparities), STAT News 2024

## Economic Opportunity (USA, 5% capture)

Scenario	Patients Found	Ada Revenue (8% fee)	Ada Revenue (12% fee)
1% capture	194	\$3.0M	\$4.5M
<b>5% capture</b>	<b>972</b>	<b>\$14.9M</b>	<b>\$22.4M</b>
10% capture	1,944	\$29.9M	\$44.8M

### Company Motivation: 10/10

- CF franchise is Vertex's "crown jewel" (\$10.5B annual revenue)
- Explicit goal to reach "all eligible CF patients globally"
- High per-patient economics (\$192K net/year) justify Patient Finder investment
- Long patent runway (2037) enables long-term partnership
- 35% global underdiagnosis aligns with market expansion positioning

**Pitch Hook:** "With 35% of cystic fibrosis patients globally undiagnosed—including thousands in the United States, particularly among minority populations experiencing diagnostic delays of up to 4 years—Ada Patient Finder can help Vertex reach more of the 90% of CF patients eligible for Trikafta. At \$192,000 net revenue per patient per year, every 200 patients Ada finds generates \$3-4.5M in Ada fees while expanding Vertex's \$10.5B CF franchise and delivering life-changing care to families who've been searching for answers to chronic respiratory and digestive symptoms."

## 2. ZIMISLECEL (VX-880 - Type 1 Diabetes Cell Therapy)

Fit Score: 9.5/10

### Market Opportunity

**USA Addressable Undiagnosed:** 7,500 patients

**Net Revenue/Patient:** \$1,000,000 (one-time, estimated)

**Ada Fee/Patient:** \$80,000 - \$120,000

**Status:** Phase 3 (FORWARD trial)

**Expected Approval:** 2027-2028

Market	Total T1D Prevalence	Estimated Undiagnosed	Hard-to-Control T1D	Drug-Addressable
USA	1.45 million	75,000-145,000	35,000-40,000	7,500
Europe	2.7 million	~135,000-270,000	~135,000-189,000	~2,000
Global	9.2-9.5 million	~460,000-950,000	~460,000-665,000	~16,000

Sources: LIV Hospital 2025 (1.45M USA), JAMA 2024, IDF Diabetes Atlas 2025, HemoCue 2025 (global data)

## Clinical Profile

**Primary Symptoms:** Classic triad of polyuria (frequent urination), polydipsia (excessive thirst), polyphagia (excessive hunger); unexplained weight loss, fatigue, blurred vision, slow-healing sores

**Diagnostic Delay:** 21% of pediatric T1D cases experience diagnostic delay, can lead to diabetic ketoacidosis (DKA) at presentation (30-40% of new diagnoses)

**Ada Surface Ability: 9/10** - Classic triad is VERY distinctive. Ada can catch early cases before DKA, flag atypical presentations (adults misdiagnosed as Type 2). Simple blood glucose testing confirms.

Source: Existing research report citing diagnostic delay studies

## Economic Opportunity (USA, 5% capture)

Scenario	Patients Found	Ada Revenue (8% fee)	Ada Revenue (12% fee)
1% capture	75	\$6.0M	\$9.0M

Scenario	Patients Found	Ada Revenue (8% fee)	Ada Revenue (12% fee)
5% capture	375	\$30.0M	\$45.0M

*Note: One-time revenue, annualized over 3-5 year market ramp = \$6M-\$15M/year*

## Company Motivation: 10/10

- T1D is Vertex's next mega-franchise target after CF
- "Potentially curative" therapy = game-changer positioning
- \$400M write-off of VX-264 to focus on Zimislecel = massive commitment
- ViaCyte acquisition \$320M, TreeFrog partnership = strategic infrastructure
- Early diagnosis → long-term patient relationships (lifelong immunosuppression monitoring)
- High per-patient economics (\$1M estimated)

*Sources: BioSpace 2024 (VX-264 discontinuation), Diabetes Research Connection 2024 (ViaCyte acquisition), FiercePharma 2024 (TreeFrog partnership)*

**Pitch Hook:** "Twenty-one percent of children with type 1 diabetes experience dangerous diagnostic delays, often presenting in life-threatening diabetic ketoacidosis. Ada Patient Finder can identify undiagnosed T1D early through classic symptom patterns—excessive thirst, frequent urination, unexplained weight loss—and connect them to care immediately. Looking ahead, these patients become candidates for Vertex's potentially curative cell therapy, Zimislecel. At an estimated \$1M net revenue per patient, every 100 patients Ada finds generates \$8-12M in Ada fees while building Vertex's next billion-dollar franchise and giving patients a path to freedom from insulin."

**Strategic Note:** Partner with Vertex NOW (pre-launch) to build database of T1D patients found by Ada, establish relationship and trust with patients, and position for Zimislecel conversion when approved (2027-2028). Early mover advantage before competitors enter cell therapy market.

### 3. INAXAPLIN (VX-147 - APOL1 Kidney Disease)

**Fit Score: 8.0/10**

#### Market Opportunity

**USA Addressable Undiagnosed:** 200,000-300,000 patients

**Net Revenue/Patient:** \$120,000/year (estimated)

**Ada Fee/Patient:** \$9,600 - \$14,400/year

**Status:** Phase 2/3 (orphan drug designation)

**Expected Approval:** 2026-2027

Population Metric	Number	Source
African Americans in USA	~47 million	US Census
Carriers (1 in 8)	~5.9 million	NephCure 2024
Two risk alleles (13% of African Americans)	~6.1 million	Duke Health 2024
Develop kidney disease (15-20%)	~1.07 million	Calculated
Diagnosed (30-40%)	~320,000-430,000	Estimated
<b>Undiagnosed (60-70%)</b>	<b>~640,000-750,000</b>	<b>90% asymptomatic early</b>
<b>Drug-Addressable Undiagnosed</b>	<b>~250,000</b>	<b>Moderate-advanced CKD</b>

Sources: NephCure Kidney International 2024, Duke Health 2024 (APOL1 genetics), NephCure 2024 (90% asymptomatic)

#### Health Disparity Context

- African Americans: **13% of US population, 35% of kidney failure cases**

- **40% of African Americans on dialysis** have APOL1-mediated kidney failure
- Black Americans experience kidney failure at **4× the rate** of other ethnic groups
- APOL1 patients progress to dialysis **10 years earlier** than others

Source: NephCure 2024, Duke Health 2024

## Clinical Profile

**Primary Symptoms:** Early stages ASYMPTOMATIC (90% no visible symptoms). Later: proteinuria, hematuria, edema, fatigue, decreased urine output, high blood pressure.

Advanced: nausea, vomiting, muscle cramps.

**Diagnostic Delay:** Significant—often diagnosed at advanced stages. Asymptomatic early disease, APOL1 testing NOT routine even when biopsy done.

**Ada Surface Ability: 6/10** - Early disease asymptomatic (cannot surface), BUT Ada can flag high-risk populations for screening: African American users with family history of kidney disease, hypertension + proteinuria/hematuria, unexplained edema/fatigue/urinary changes. Ada's value: Risk-based screening prompts rather than symptom surfacing.

## Economic Opportunity (USA, 5% capture)

Scenario	Patients Found	Ada Revenue (8% fee)	Ada Revenue (12% fee)
1% capture	2,500	\$24.0M	\$36.0M
<b>5% capture</b>	<b>12,500</b>	<b>\$120.0M</b>	<b>\$180.0M</b>

## Company Motivation: 9/10

- Addresses massive unmet need and health disparity
- 35% of kidney failure in 13% of population = enormous burden
- First APOL1-specific therapy = market leadership
- Orphan drug status = pricing power
- High per-patient economics (\$120K/year net)
- Chronic disease = recurring revenue
- Perfect Patient Finder fit for early detection → slow progression

**Pitch Hook:** "African Americans represent 13% of the US population but 35% of kidney failure cases—and 40% of those on dialysis have APOL1-mediated kidney disease, often diagnosed only after irreversible damage. Ada Patient Finder can identify high-risk individuals early through family history, subtle symptoms like unexplained swelling or fatigue, and prompt APOL1 genetic testing and urinalysis. At \$120,000 net revenue per patient per year, every 200 patients Ada finds generates \$1.9-2.9M in annual Ada fees while addressing one of the most pressing health disparities in America and expanding Vertex's pipeline into nephrology."

**Strategic Note:** Partner pre-launch to build awareness of APOL1 testing in African American communities, position Ada as health equity solution, create database of high-risk patients for Inaxaplin launch, collaborate with nephrology societies and patient advocacy groups.

## TIER 2: Strong Fit Assets

### 4. CASGEVY (Sickle Cell Disease & Beta Thalassemia)

**Fit Score: 7.5/10**

#### Market Opportunity

**USA Addressable Undiagnosed:** ~2,925 patients (SCD + beta-thal)

**Net Revenue/Patient:** \$1,650,000 (one-time)

**Ada Fee/Patient:** \$132,000 - \$198,000

**2025 Revenue:** \$116M (launch year)

**Patent Expiry:** 2034 (foundational CRISPR patents)

**USA Prevalence:** ~100,000 SCD patients, 1,200-2,100 beta-thal patients

**Underdiagnosis Evidence:** SCD mortality rates 11× higher than official statistics (massive underestimation). Despite newborn screening, adult SCD patients may be undiagnosed (missed screening, immigrant populations, mild phenotypes).

Sources: GoodRx 2025 (100K SCD), DelveInsight 2025 (beta-thal), Existing research report (mortality underestimation)

## Economic Opportunity (USA, 5% capture)

**Combined Opportunity:** 146 patients × \$132K-198K = \$19.3M - \$28.9M (one-time)  
Annualized over 3-year ramp: \$6.4M - \$9.6M per year

### Scoring

**Ada Surface Ability: 7/10** - Moderate surfacing for SCD (pain crises + anemia + jaundice + infections). Good surfacing for beta-thal (severe anemia + growth delays). Family history, ethnic background enhance signal.

**Company Motivation: 8/10** - Gene therapy is strategic priority. Slow uptake (\$116M vs blockbuster CF) creates motivation for market expansion. High per-patient economics justify Patient Finder. BUT: Reimbursement challenges and outcomes-based contracts complicate model.

**Pitch Hook:** "Sickle cell disease mortality is 11 times higher than official statistics, revealing a massive hidden patient burden. Ada Patient Finder can help Vertex identify adults with undiagnosed or undertreated sickle cell disease and beta thalassemia—particularly in immigrant populations and those who fell through newborn screening gaps—and connect them to Casgevy's one-time curative gene therapy. At \$1.65M net revenue per patient, every 10 patients Ada finds generates \$1.3-2M in Ada fees while transforming lives with a potential cure."

## 5. POVETACICEPT (ALPN-303 - IgA Nephropathy)

**Fit Score: 8.0/10**

### Market Opportunity

**USA Addressable Undiagnosed:** 15,000-20,000 patients

**Net Revenue/Patient:** \$105,000/year (estimated)

**Ada Fee/Patient:** \$8,400 - \$12,600/year

**Status:** Phase 3 (Vertex acquired Alpine for \$4.9B)

**Expected Approval:** 2026-2027

**USA IgAN Prevalence:** ~100,000-150,000 total | Incidence: 1.4 per 100,000 person-years

**Underdiagnosis:** 30-40% (diagnosed at advanced stages, asymptomatic early presentation)

Sources: Kaiser Permanente 2023, Academic OUP 2024, Existing research report

## Economic Opportunity (USA, 5% capture)

Scenario	Patients Found	Ada Revenue (8% fee)	Ada Revenue (12% fee)
1% capture	175	\$1.47M	\$2.21M
<b>5% capture</b>	<b>875</b>	<b>\$7.35M</b>	<b>\$11.03M</b>

## Competitive Context

**Direct Competitor:** Atacicept (Vera Therapeutics) - BLA submission Q4 2025 (same timeline as povetacept)

**Atacicept Phase 3 Results:** 45.7% proteinuria reduction vs 6.8% placebo (P<0.001)

**Implication:** Competitive urgency—early diagnosis = competitive advantage. "Win the market by finding patients first."

Sources: PubMed 2025 (Atacicept ORIGIN 3 results), CheckRare 2025

## Scoring

**Ada Surface Ability: 6/10** - Early asymptomatic disease, BUT Ada can surface hematuria (especially post-URI), proteinuria (foamy urine), edema + urinary symptoms. Pattern recognition: Hematuria + recent cold/URI = classic IgAN signal.

**Company Motivation: 9/10** - \$4.9B acquisition = massive commitment. "Pipeline in a product" (multiple indications beyond IgAN). Lead asset in immunology franchise. High per-patient economics. Chronic disease = recurring revenue. Competitive market creates urgency.

**Pitch Hook:** "IgA nephropathy is a silent disease, often diagnosed only after irreversible kidney damage. Many patients with persistent blood in their urine—"

*especially after colds or respiratory infections—dismiss it or receive inadequate follow-up. Ada Patient Finder can identify these early warning signs, prompt simple urinalysis, and connect patients to nephrology before progression to kidney failure. With Vertex's \$4.9 billion investment in povetacicept and a \$105,000 net revenue per patient per year, every 100 patients Ada finds generates \$840K-\$1.26M in annual Ada fees while building Vertex's immunology franchise and preserving kidney function for thousands."*

## TIER 3: Lower Priority / Conditional Fit

### Tier 3 Assets Summary

Drug	Indication	Fit Score	USA Addressable	Ada Opportunity (5% capture)	Key Limitation
<b>Vanazalanib</b>	ADPKD	5.5/10	20,000	\$6M - \$9M/yr	Lower strategic priority vs Inaxaplin/Povetacicept; early asymptomatic disease
<b>VX-522</b>	AAT Deficiency	6.5/10	79,000	\$37.9M - \$56.9M/yr	Phase 2 (earlier stage); less strategic prominence than Tier 1-2 assets

**Recommendation:** Consider Tier 3 assets only if Tier 1-2 partnerships secured. VX-522 has larger opportunity (79K addressable) but lower strategic priority. Both are pre-launch (2027-2028 estimated approvals).

# NO: Poor Fit Assets

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## Assets Not Suitable for Patient Finder

### JOURNAVX (Suzetrigine - Acute Pain)

Fit Score: 1.0/10

#### Why NO:

- Acute pain is NOT undiagnosed (self-evident symptom)
- Mass market, not rare disease
- Low per-patient revenue (\$50-200 estimated) and short treatment duration (days)
- Ada PF fee would be \$12-18 per patient (far below economic threshold)
- Patient Finder model assumes: (1) undiagnosed patients exist, (2) high per-patient revenue, (3) long treatment duration
- **Journavx fails ALL criteria**

**Recommendation:** Vertex should pursue traditional DTC marketing, physician detailing, and payer contracts for Journavx—NOT patient finding. This is a treatment navigation opportunity, not a patient finding opportunity.

### KALYDECO / ORKAMBI / SYMDEKO (Older CF Drugs)

Fit Score: 2.0/10

#### Why NO:

- All three being **replaced by Trikafta** (superior, broader indication)
- Patent expiry 2026 (Kalydeco, Orkambi) - generic competition imminent
- Declining revenue - not strategic focus
- Same undiagnosed patient pool as Trikafta - why find patients for inferior products?
- Vertex actively switching patients TO Trikafta

**Recommendation:** Any CF patient finding efforts should focus exclusively on Trikafta/Kaftrio. Do not pitch these sunset assets separately.

# Strategic Recommendations

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## Prioritization Framework

### Immediate Priority (Tier 1):

1. **Trikafta** - Largest revenue, mature product, proven market, immediate value
2. **Inaxaplin** - Massive undiagnosed population, health equity angle, pre-launch advantage
3. **Zimislecel** - Pre-launch database building, long-term franchise potential

### Strong Secondary (Tier 2):

4. **Povetacicept** - Competitive urgency (vs Atacicept), pre-launch timing
5. **Casgevvy** - High per-patient economics, slow uptake = market expansion need

### Consider if Capacity Allows (Tier 3):

6. **VX-522** - Large undiagnosed population but lower strategic priority
7. **Vanazalanib** - Moderate opportunity, less strategic prominence

## Recommended Lead Pitch: Bundle Trikafta + Inaxaplin

- **Trikafta:** Proven revenue, immediate value, mature product
- **Inaxaplin:** Impact/equity story, massive undiagnosed population (250K), pre-launch timing
- **Combined USA opportunity (5% capture):** \$134.9M - \$202.4M annually
- **Narrative:** "Market expansion for Vertex's crown jewel CF franchise + addressing one of America's most pressing health disparities"

## Partnership Phasing Strategy

Phase	Assets	Timeline	Rationale
<b>Phase 1</b>	<b>Trikafta + Inaxaplin</b>	<b>Launch 2026</b>	<b>Immediate revenue (Trikafta) + pre-launch positioning (Inaxaplin 2026-2027 approval)</b>
<b>Phase 2</b>	Add Zimislecel	2026-2027	Pre-launch partnership to build T1D patient database for 2027-2028 approval
<b>Phase 3</b>	Povetacicept + Casgevy	2027	Expand into immunology (Povetacicept) and gene therapy (Casgevy uptake acceleration)
<b>Phase 4</b>	Tier 3 (VX-522, Vanazalanib)	2027-2028	If capacity allows and Tier 1-2 partnerships performing

## Key Strategic Angles

### 1. Health Equity Positioning (Inaxaplin):

- 40% of Black Americans on dialysis have APOL1 disease
- Ada Patient Finder as solution to massive health disparity
- Resonates with ESG/impact investment priorities
- Collaboration with nephrology societies, patient advocacy groups

### 2. Franchise Building (Zimislecel):

- Partner NOW (pre-launch) to build T1D patient database
- Establish relationship and trust with patients
- Position for Zimislecel conversion when approved (2027-2028)
- Early mover advantage before competitors enter cell therapy market

### 3. Competitive Urgency (Povetacicept):

- Atacicept (Vera) on same timeline (BLA Q4 2025)
- Early diagnosis = competitive advantage
- "Win the market by finding patients first"

- Partner pre-launch to build early diagnosis pathway

## Total Addressable Opportunity

Tier	Assets	USA Addressable Undiagnosed	Annual Ada Revenue (5% capture, Low)	Annual Ada Revenue (5% capture, High)
Tier 1	Trikafta, Zimislecel*, Inaxaplin	276,940	\$140.9M - \$149.9M	\$211.4M - \$247.4M
Tier 2	Casgevy*, Povetacicept	20,425	\$13.75M	\$20.63M
Tier 3	VX-522, Vanazalanib	99,000	\$43.9M	\$65.9M
<b>TOTAL (All Tiers)</b>	<b>All viable assets</b>	<b>396,365</b>	<b>\$198.55M - \$207.55M</b>	<b>\$297.93M - \$333.93M</b>

\* Zimislecel and Casgevy are one-time revenue therapies; figures annualized over 3-5 year market ramp

### Conservative Base Case (Tier 1 + 2, 5% capture):

**\$154.65M - \$168.53M annually (Low) | \$232.03M - \$268.03M annually (High)**

This represents the most realistic near-term opportunity focusing on Vertex's highest-priority assets with proven Patient Finder fit.

This analysis is based on publicly available information, web research, and estimates where specific data is unavailable. All revenue projections are illustrative and subject to actual market conditions, regulatory approvals, and partnership terms.