

Sobi (Swedish Orphan Biovitrum) - Ada Patient Finder Analysis

v4 Comprehensive Suitability Assessment | March 2026 | Prepared by Ada Cockpit

Ada Patient Finder identifies undiagnosed patients through symptom assessment and navigates them to care.

Revenue Model: 8-12% of first-year drug revenue per patient found.

Positioning: Market expansion (not DTC marketing budget).

Executive Summary

TOTAL DRUGS ANALYZED

13

TIER 1 (PURSUE AGGRESSIVELY)

3

TIER 2 (PURSUE)

2

COMBINED ADA REVENUE
(GLOBAL, 5%)

\$703k-4.6M/yr

Drug	Indication	Tier	Fit Score	Addressable Undiagnosed (USA)	Ada Revenue Opportunity (USA, 5% capture)
Gamifant (emapalumab)	MAS in Still's Disease	TIER 1	8/10	200-500 patients	\$126k-708k/year
Tryngolza (olezarsen)	Familial Chylomicronemia Syndrome (FCS)	TIER 1	7/10	100-300 patients	\$120k-1.08M/year
Gamifant (emapalumab)	Primary HLH	TIER 1	7/10	50-150 patients	\$32k-212k/year

Drug	Indication	Tier	Fit Score	Addressable Undiagnosed (USA)	Ada Revenue Opportunity (USA, 5% capture)
Aspaveli (pegcetacoplan)	PNH	TIER 2	6/10	200-400 patients	\$160k-1.01M/year
Kineret (anakinra)	Still's Disease	TIER 2	6/10	500-1,000 patients	\$10k-19k/year
Doptelet (avatrombopag)	ITP	TIER 3	5/10	Limited	~\$32k-212k/year
Aspaveli (pegcetacoplan)	C3 Glomerulopathy	TIER 3	4/10	Small	~\$8k-54k/year
Altuvoct/Elocta/Alprolix	Hemophilia A/B	NO	4/10	Patients already in specialist care; acute presentations bypass symptom checkers	
Orfadin (nitisinone)	Hereditary Tyrosinemia Type 1	NO	3/10	Newborn screening captures nearly all cases	
NASP	Uncontrolled Gout (Pipeline)	NO	3/10	Treatment failure in diagnosed patients, not a diagnostic issue	
Vonjo (pacritinib)	Myelofibrosis	NO	2/10	SEK 6.6B impairment; lab-driven diagnosis; declining revenue	
Pozdeutinurad (AR882)	Tophaceous Gout (Pipeline)	NO	2/10	Treatment optimization, not patient finding	
Beyfortus (nirsevimab)	RSV Prophylaxis	NO	1/10	Royalty product only; not an undiagnosed population issue	
Gamifant (emapalumab)	IDS Sepsis (Pipeline Phase 2a)	MONITOR	TBD	Phase 2a data Jan 2026; massive potential market if successful	

1. Gamifant (emapalumab) - MAS in Still's Disease

Section A: Market Numbers

Drug-Addressable Funnel (USA)

Still's Disease prevalence (sJIA + AOSD): ~3,100-3,940 patients

↓ MAS incidence: 10% fulminant, 30% subclinical

↓ Undiagnosed (20-30% underdiagnosis): 627-1,048 MAS-eligible patients

↓ Gamifant-eligible (inadequate glucocorticoid response, 30-50%): **200-500 patients**

Market	Addressable Undiagnosed	Net Revenue/Patient/Year	PF 1% Capture (Ada Fee)	PF 5% Capture (Ada Fee)	PF 10% Capture (Ada Fee)
USA	200-500	\$157k-236k	\$25k-142k	\$126k-708k	\$252k-1.42M
Germany (DACH)	24-74	\$295k-334k	\$5k-30k	\$42k-221k	\$85k-437k
ROW	100-200	\$250k-350k	\$20k-84k	\$100k-420k	\$200k-840k

Pricing: WAC ~\$393,600/year (adult). Net after 40-60% gross-to-net: \$157k-236k/year (USA). EU net after 15-25% discount: \$295k-334k/year.

Annual Revenue (FY 2025): SEK ~3.05 billion (~\$287M USD, annualized from Q4 2025 SEK 763M, +49% YoY)

Peak Revenue Target: >SEK 10 billion by 2030 (\$943M USD)

Patent/Exclusivity: MAS/Still's orphan exclusivity expires June 2032. Primary HLH exclusivity expired Nov 2025.

Competitors: Novartis (ruxolitinib off-label, MAS825 in trials), AB2 Bio (tadekinig alfa, late-stage), Electra Therapeutics (ELA026, late-stage). Gamifant holds 34.6% HLH treatment market share.

Section B: Clinical & Diagnostic Profile

- **Key Symptoms:** Quotidian fever (daily spikes to 39-40C returning to normal), salmon-pink evanescent rash (appears with fever, fades when fever resolves), polyarthralgia/arthritis, sore throat, hepatosplenomegaly

- **Diagnostic Delay:** Common; quotidian fever misattributed to viral infection due to low clinician awareness
- **Common Misdiagnoses:** Viral infection (fever + rash), drug reaction, other autoinflammatory conditions
- **Who Diagnoses:** Rheumatologists (AOSD/sJIA), immunologists
- **Ada Surface Ability: 9/10** - Quotidian fever + evanescent rash + arthralgia is highly distinctive and surfaceable. Ada can capture the temporal relationship (fever spike, rash appearance, both resolve daily).
- **Treatment:** Second/third-line for refractory MAS (after glucocorticoids). Induction therapy (IV twice weekly).

Section C: Commercial Signals

- New MAS/Still's FDA approval June 2025 creating immediate market education needs
- Peak sales target >SEK 10B requires 3.3x growth by 2030 (currently ~\$287M annual)
- Gamifant designated as one of six major launches driving SEK 55B 2030 revenue ambition
- Diagnostic delay and misdiagnosis (fever as infection) is documented barrier
- **Actively looking for patients: YES (strong signals)**

Section D: Opportunity Assessment

ADA SURFACE ABILITY

9/10

COMPANY MOTIVATION

9/10

OVERALL FIT SCORE

8/10

Pitch Hook: Still's disease patients endure diagnostic delays as the signature quotidian fever (daily spikes to 39-40C) and evanescent salmon-pink rash are routinely misattributed to viral infections. Up to 30% of sJIA patients develop MAS, a life-threatening hyperinflammatory complication with near-uniform fatality if untreated. Gamifant, FDA-approved June 2025 as the first and only MAS therapy in Still's, is a \$394k/year biologic that requires early identification of both underlying Still's and MAS onset. Ada's symptom checker captures the highly distinctive quotidian fever pattern and temporal rash relationship to flag rheumatology referral and MAS risk monitoring, potentially identifying 200-500 Gamifant-eligible patients in the US and generating \$126k-708k in annual Ada revenue at 5% capture.

2. Tryngolza (olezarsen) - Familial Chylomicronemia Syndrome (FCS)

TIER 1 - PURSUE AGGRESSIVELY

Fit Score: **7/10**

Section A: Market Numbers

Drug-Addressable Funnel (USA)

FCS prevalence: 335-3,350 patients (1:100k to 1:1M)

↓ Conservative estimate: 335-670 patients

↓ Undiagnosed (40-60%, given 24-year median delay): 168-335 patients

↓ Tryngolza-eligible (genetically confirmed, treatment-ready, 80-90%): **100-300 patients**

Market	Addressable Undiagnosed	Net Revenue/Patient/Year	PF 1% Capture (Ada Fee)	PF 5% Capture (Ada Fee)	PF 10% Capture (Ada Fee)
USA	100-300	\$300k-600k	\$24k-216k	\$120k-1.08M	\$240k-2.16M
Germany (DACH)	30-90	\$300k-600k	\$7k-65k	\$36k-324k	\$72k-648k
ROW	21-68	\$200k-400k	\$3k-33k	\$16k-144k	\$34k-327k

Pricing: Estimated WAC ~\$700k-1.5M/year. Net after 40-60% gross-to-net: \$300k-600k/year.

Annual Revenue: 2025 US net sales \$105-108M (Ionis, first launch year). Q4 2025 \$50M (+56% QoQ).

Peak Revenue: >\$2 billion (Ionis guidance, raised from >\$1B, driven by sHTG/MCS expansion)

Patent/Exclusivity: US FCS orphan exclusivity through Dec 2031. EU orphan exclusivity through Jan 2036. ASO patents likely extend 2035-2040+.

Competitors (FCS): None (first-in-class). No approved FCS therapies prior to Tryngolza.

Section B: Clinical & Diagnostic Profile

- **Key Symptoms:** Recurrent acute pancreatitis (severe epigastric pain), eruptive xanthomas, extreme hypertriglyceridemia (>1,000 mg/dL, often >2,000-5,000), fatty food intolerance, milky

plasma

- **Diagnostic Delay:** Median **24 years** from symptom onset. Average **5 physicians** consulted before diagnosis.
- **Common Misdiagnoses:** Multifactorial chylomicronemia syndrome (MCS, 1:600 prevalence), poorly controlled diabetes, familial combined hyperlipidemia, idiopathic recurrent pancreatitis
- **Who Diagnoses:** Endocrinologists, lipidologists, gastroenterologists (pancreatitis workup), geneticists
- **Ada Surface Ability: 7/10** - Recurrent pancreatitis in young patients (<40 years) + family history of high triglycerides is highly suggestive. Ada can differentiate from general pancreatitis causes (alcohol, gallstones).
- **Treatment:** First-line FCS treatment. Lifelong SC injection every 4 weeks. Replaces inadequate fat-restricted diet/fibrates.

Section C: Commercial Signals

- US FDA approval Dec 2024; EU approval Jan 2026 (Sobi commercializes ex-US)
- Revenue trajectory strong: \$50M Q4 2025 (+56% QoQ sequential growth)
- Ionis raised peak sales guidance to >\$2B (from >\$1B)
- Language emphasizes "underdiagnosed population" and "significant unmet need"
- FCS partnership could pilot for much larger sHTG/MCS patient finding (~700k EU-5 patients)
- **Actively looking for patients: YES (moderate to strong signals)**

Section D: Opportunity Assessment

ADA SURFACE ABILITY

7/10

COMPANY MOTIVATION

8/10

OVERALL FIT SCORE

7/10

Pitch Hook: FCS patients endure a 24-year median diagnostic odyssey, consulting an average of 5 physicians, as recurrent pancreatitis is routinely misattributed to alcohol, gallstones, or MCS (1:600 prevalence vs. FCS at 1:100k-1M). Most cases manifest in childhood with recurrent severe abdominal pain but aren't diagnosed until adulthood after irreversible pancreatic damage. Tryngolza, the first and only approved FCS therapy (\$300-600k/year net), requires genetic confirmation, creating a classic patient-finding challenge. Ada surfaces the distinctive pattern of young patients with recurrent unexplained pancreatitis + family history of extreme hypertriglyceridemia + no secondary causes, shortening the 24-year odyssey and capturing an estimated 100-300 undiagnosed US patients worth \$120k-1.08M in annual Ada revenue at 5% capture.

3. Gamifant (emapalumab) - Primary HLH

TIER 1 - PURSUE AGGRESSIVELY

Fit Score: **7/10**

Section A: Market Numbers

Drug-Addressable Funnel (USA)

Primary HLH incidence: ~33-67 new cases/year (1 in 50,000-100,000 births)

↓ Undiagnosed (25-40%): 8-27 new cases/year

↓ Prevalent pool (living with HLH): ~50-150 patients

↓ Gamifant-eligible (refractory/recurrent, 60-80%): **50-150 patients**

Market	Addressable Undiagnosed	Net Revenue/Patient/Year	PF 1% Capture (Ada Fee)	PF 5% Capture (Ada Fee)
USA	50-150	\$157k-236k	\$6k-42k	\$32k-212k
Germany (DACH)	12-35	\$295k-334k	\$3k-14k	\$14k-84k
ROW	50-100	\$250k-350k	\$10k-42k	\$10k-50k

- **Diagnostic Delay:** Median ~1 month. Nearly uniformly fatal if untreated.
- **Ada Surface Ability: 7/10** - Constellation of persistent high fever + cytopenias (fatigue, pallor, bleeding) + hepatosplenomegaly. Less specific than Still's but pattern can flag hyperinflammatory syndrome.
- **Common Misdiagnoses:** Sepsis, overwhelming infection, autoimmune disease, malignancy-related syndromes
- **Note:** Bundle with MAS/Still's in same Gamifant partnership discussion.

4. Aspaveli (pegcetacoplan) - PNH

TIER 2 - PURSUE

Fit Score: **6/10**

Market	Addressable Undiagnosed	Net Revenue/Patient/Year	PF 5% Capture (Ada Fee)
USA	200-400	\$200k-420k	\$160k-1.01M
Germany (DACH)	30-75	\$375k-595k	\$45k-268k
ROW	2-15	\$300k-500k	\$2k-90k

- **Diagnostic Delay:** Median ~2 years. <40% diagnosed within 12 months. 40% experience thrombosis before diagnosis.
- **Ada Surface Ability: 6/10** - Dark urine (hemoglobinuria, especially morning) is pathognomonic and surfaceable. Fatigue + thrombosis in unusual sites adds specificity.
- **Limitation:** Aspaveli competes with established C5 inhibitors (Soliris, Ultomiris) and oral Fabhalta. PNH market relatively mature.
- **Best approach:** Bundle with C3G (first-approved therapy, EU Jan 2026) in Aspaveli partnership discussion.

***Pitch Hook:** PNH patients wait a median 2 years for diagnosis, with 40% suffering life-threatening thrombosis before identification, because the pathognomonic dark morning urine (hemoglobinuria) is misattributed to UTI or kidney disease. Ada surfaces this distinctive pattern, combined with fatigue and unexplained thrombosis history, to trigger urgent hematology referral for flow cytometry.*

Drugs Assessed as NOT Suitable

Drug	Indication	Fit Score	Reason
Doptelet	ITP	5/10	Low diagnostic delay; lab-dependent diagnosis (CBC); narrow drug-eligible funnel (chronic/refractory only)
Aspaveli	C3 Glomerulopathy	4/10	Kidney biopsy required for diagnosis; nonspecific symptoms overlap with many kidney diseases; very small population

Drug	Indication	Fit Score	Reason
Altuvoct/Elocta/Alprolix	Hemophilia A/B	4/10	Drug-eligible patients already managed by hematologists; acute presentations require ER, not symptom checker
Orfadin	Hereditary Tyrosinemia Type 1	3/10	Newborn screening captures nearly all cases (81% EU NBS programs screen for HT-1)
NASP	Uncontrolled Gout (Pipeline)	3/10	Treatment failure in already-diagnosed patients; not a diagnostic issue
Vonjo	Myelofibrosis	2/10	SEK 6.6B impairment (Nov 2025); lab-driven diagnosis; declining revenue (-8% CER FY 2025)
Pozdeutinurad	Tophaceous Gout (Pipeline Phase 3)	2/10	Treatment optimization in diagnosed patients, not patient finding
Beyfortus	RSV Prophylaxis	1/10	Royalty product only (AstraZeneca/Sanofi); all infants eligible; not an undiagnosed population issue
Gamifant	IDS Sepsis (Pipeline Phase 2a)	TBD	Phase 2a EMBRACE trial data Jan 2026; massive potential market if Phase 3 successful; monitor pipeline

Strategic Recommendation

Immediate Partnership Priority

- Gamifant (MAS/Still's + HLH bundle)** - Highest fit (8/10), new indication (Jun 2025), most distinctive symptoms, >SEK 10B peak sales target. Lead with MAS, include primary HLH as bonus.
- Tryngolza (FCS)** - Extreme 24-year diagnostic delay, first-in-class monopoly, EU launch Jan 2026 creates immediate need. Pilot for scaling to sHTG (~700k EU-5 patients).
- Aspaveli (PNH + C3G bundle)** - PNH has surfaceable dark urine symptom; C3G is first-approved therapy. Bundle for Aspaveli franchise discussion.

Revenue Summary

Scenario	USA Only	Global (USA + DACH + ROW)
Tier 1 drugs at 5% capture	\$278k-2.0M/year	\$496k-3.25M/year
Tier 1+2 drugs at 5% capture	\$448k-3.03M/year	\$703k-4.62M/year
Tier 1 drugs at 10% capture	\$555k-4.0M/year	\$992k-6.5M/year

Why Sobi Now

- **Timing:** Three new launches in 12 months (Gamifant MAS Jun 2025, Tryngolza EU Jan 2026, Aspaveli C3G Jan 2026)
- **Growth pressure:** SEK 55B 2030 target requires doubling revenue from 2025; patient finding is growth lever
- **Rare disease focus:** Entire portfolio is rare/specialty with high underdiagnosis rates and high per-patient drug revenue
- **Documented unmet need:** 24-year delays (FCS), 2-year delays (PNH), weeks-months (HLH/MAS)
- **First-in-class advantages:** Gamifant (MAS) and Tryngolza (FCS) are first/only approved therapies; every found patient = their patient

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Analysis based on public sources including Sobi Q4 2025 report, Capital Markets Day 2026, FDA/EMA databases, peer-reviewed literature, and industry analyst reports. 110+ sources cited in full report. All revenue estimates use gross-to-net discounts (US: 40-60%, EU: 15-25%).