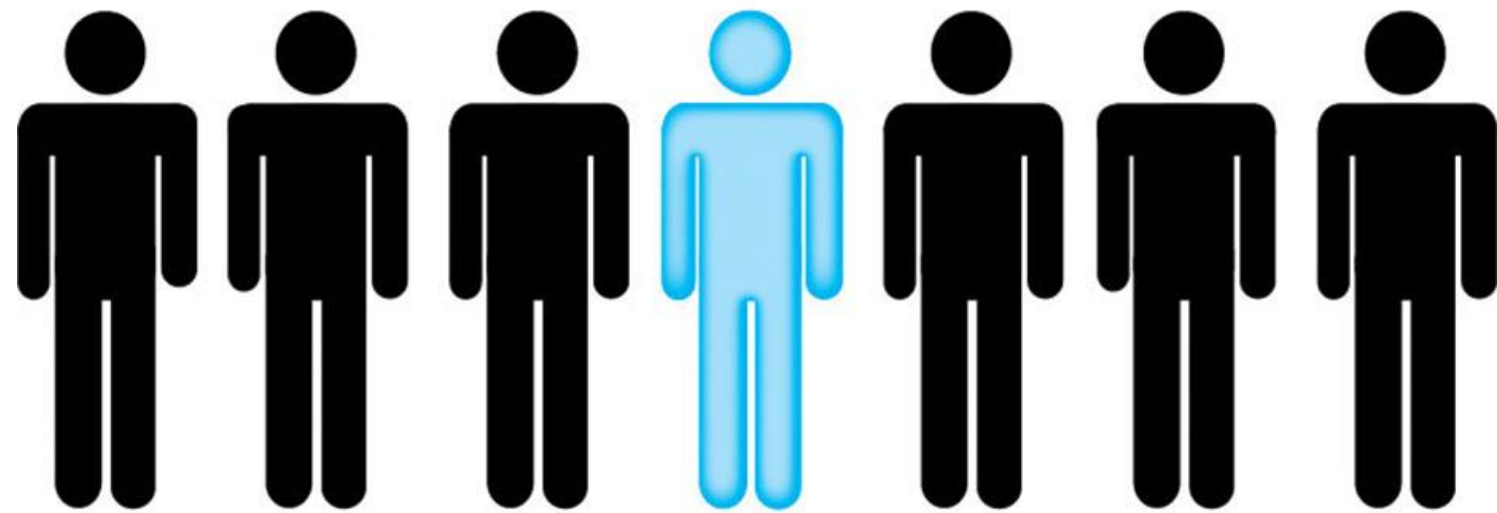


# Rare Disease Treatment Market

Industry Forecast and Outlook



RARE DISEASES



# Rare Disease Treatment Market: Industry Forecast and Outlook

\$XXB

Global Market Value

2025 rare disease treatment market valuation

13.1%

Market CAGR

Projected growth rate through 2030

\$XXB

2030 Projection

Expected market size under optimal conditions

The global rare disease treatment market demonstrates exceptional growth potential, with the **complement inhibitors submarket** leading the charge at an even more impressive 13.1% CAGR (2025–2032), expanding from USD XX billion in 2025 to USD XX billion by 2032. This acceleration is driven by rising prevalence of complement-mediated diseases and robust pipeline innovation.

## Advanced Diagnostics

Increasing diagnosis rates through genomic screening and AI-powered diagnostic tools are identifying more patients earlier in disease progression.

## R&D Investment Surge

Strong investment in biologics, gene therapies, and small molecule inhibitors is accelerating therapeutic development timelines.

## Regulatory Support

Orphan drug designation and accelerated approval pathways are reducing time-to-market for breakthrough therapies.

## Indication Expansion

New therapeutic targets including age-related macular degeneration, lupus nephritis, and IgA nephropathy are broadening market opportunities.

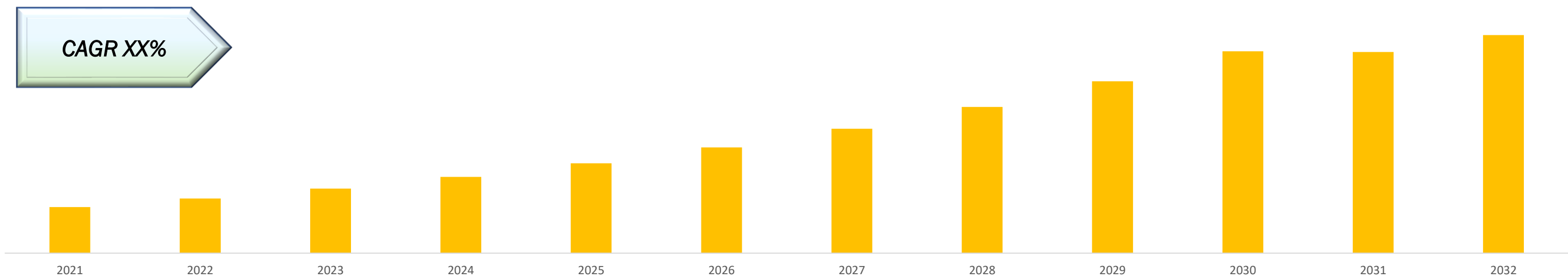
# Pipeline Breakthroughs: Recent FDA Approvals and Market Entries



These approvals represent a fundamental evolution in complement inhibition strategy, moving from exclusively targeting the terminal pathway (C5) to upstream intervention points (C3, Factor B, Factor D, C1s), offering patients more targeted and convenient treatment options.

# Global Rare Disease Treatment Market Size & Forecast

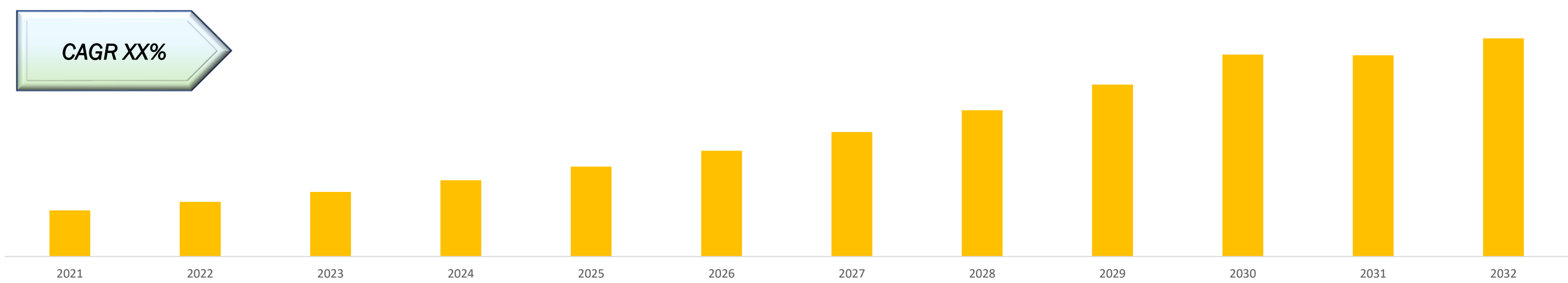
- Global Rare Disease Treatment Market Size, 2021-2032



- The escalating cost of fuel has made gasoline-powered two-wheelers increasingly expensive to operate. This economic pressure is a paramount driver for the adoption of electric scooters (E2Ws), which offer significantly lower running costs.
- In India, where two-wheelers make up over 70% of the vehicle fleet, the combination of rising fuel prices, lower operating costs, and government subsidies through programs like FAME-II has made E2Ws a financially compelling choice for both individual consumers and the rapidly growing last-mile delivery sector.
- Fleet operators, in particular, are prioritizing E2Ws for their high mileage and lower cost per kilometer.

# Regional Rare Disease Treatment Market Size & Forecast

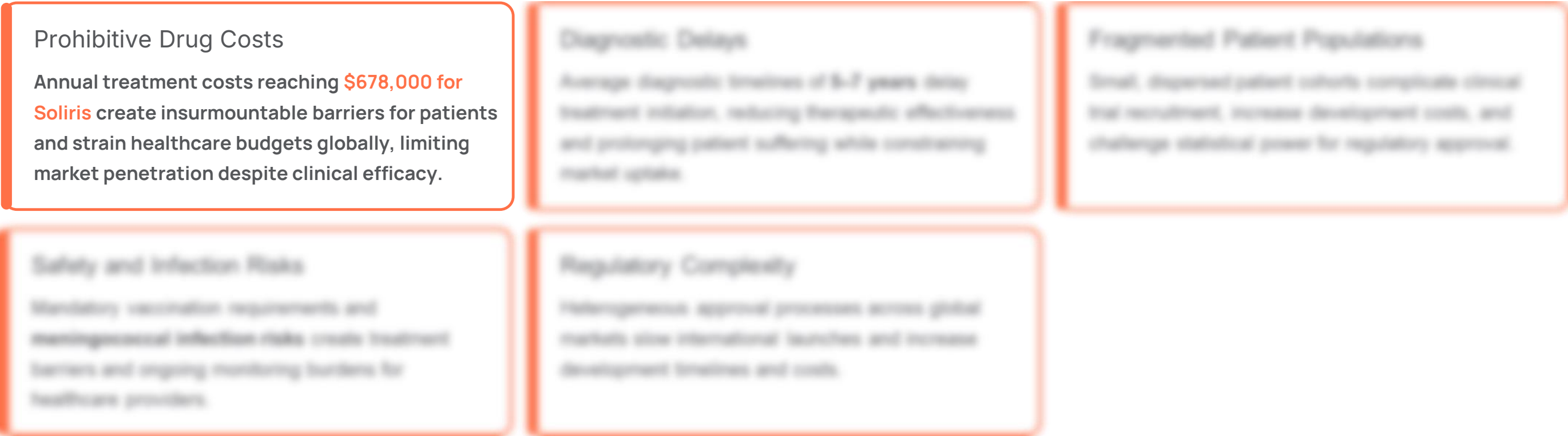
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# Industry Growth Barriers: Critical Challenges Ahead

Despite unprecedented market momentum, the complement inhibitors industry confronts substantial obstacles that threaten to constrain growth potential and patient access. These challenges span economic, regulatory, clinical, and operational domains.



## Market Access Challenges

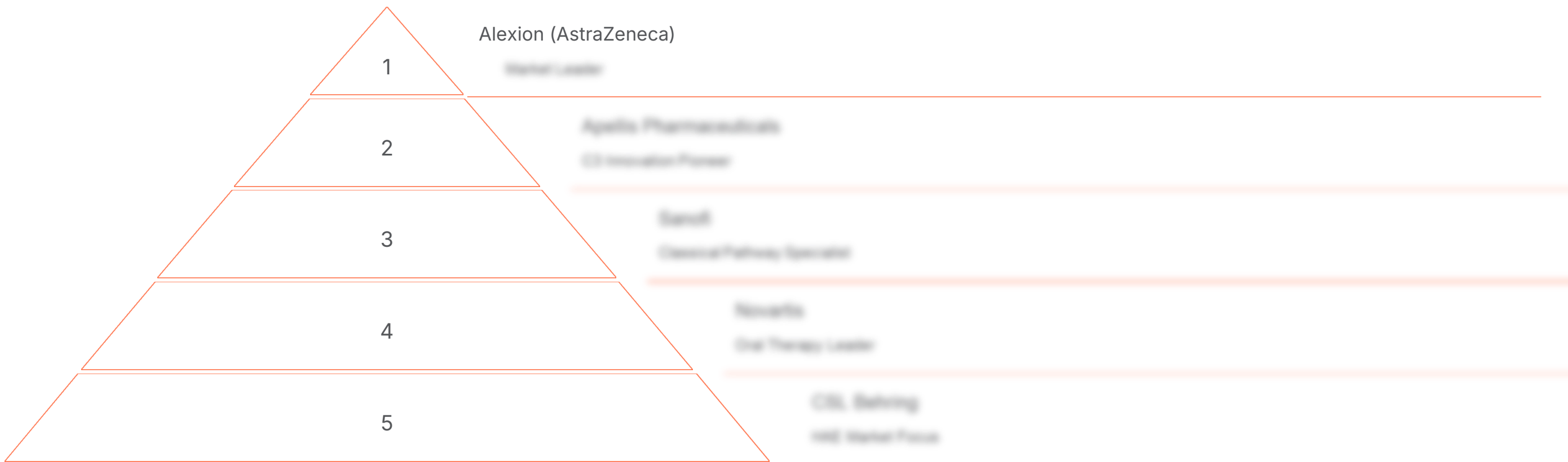
- Biosimilar competition eroding legacy drug market share
- Reimbursement hurdles in public and private payer systems
- Limited long-term efficacy data affecting payer confidence

## Operational Constraints

- Manufacturing complexity limiting biologics scalability
- Intellectual property risks from patent expirations
- Legal challenges threatening market exclusivity

# Market Concentration: A Highly Competitive Landscape

The complement inhibitors market exhibits **high concentration** among a select group of pharmaceutical leaders, with each company leveraging distinct competitive advantages and therapeutic approaches to capture market share in this rapidly expanding sector.



This concentrated market structure reflects the high barriers to entry in complement inhibition, including complex manufacturing requirements, extensive clinical development costs, and the need for specialized expertise in rare disease commercialization. The leading companies have established strong intellectual property portfolios and regulatory relationships that create significant competitive moats.

📌 Market concentration is expected to intensify as biosimilar competition and patent expirations force consolidation among smaller players, while established leaders expand their pipelines through strategic acquisitions and partnerships.

# Competitive Intelligence: Major Players and Strategic Platforms

Company	Key Products	Mechanism	Target Indications	Competitive Advantage
Alexion (AstraZeneca)	Soliris, Ultomiris, Enjaymo	C5, C1s inhibition	PNH, aHUS, gMG, NMOSD, CAD	First-mover advantage, global footprint, extended dosing intervals
Apellis Pharmaceuticals	Enprolone (upstream C3)	C3 inhibition	PNH, Idiopathic Thrombocytopenic Purpura	Smaller molecule molecule, subcutaneous self-administration
Novartis	Ultomiris (C1s)	Factor B inhibition	PNH, IgA, C3G, LAL	Oral administration, combination therapy potential
Sanofi	Enprolone (classical)	C1s inhibition	CAD	First-in-class classical pathway inhibition
CSL Behring	Berinert, HAD-IG, HAD-IG-IG	C1-INH replacement	Hereditary Angioedema	Subcutaneous options, competitive pricing
Rallybio	RL-101 (C3)	Anti-C3 mAb	PNH, gMG	Extended dosing and safety profile potential

The competitive landscape reveals distinct strategic approaches: Alexion leverages its established C5 inhibitor franchise and global infrastructure, while Apellis differentiates through upstream C3 targeting. Novartis pioneers oral convenience with factor B inhibition, and Sanofi focuses on classical pathway specialization. CSL Behring maintains its hereditary angioedema stronghold through subcutaneous delivery innovations.

## Market Access Strategy

Companies are investing heavily in health economics outcomes research and real-world evidence generation to support payer negotiations and demonstrate long-term value propositions.


## Pipeline Diversification

Leading players are expanding beyond traditional indications into ophthalmology, nephrology, and neurology to maximize platform value and reduce concentration risk.




# Alexion's Market Dominance Under Siege

Alexion maintains its position as the **dominant player in the C5 inhibitor space**, with Soliris and Ultomiris generating combined annual sales exceeding \$6 billion. However, this market leadership faces unprecedented challenges from multiple competitive vectors threatening both pricing power and market share.




Next-Generation C5 Inhibitors

Improved dosing regimens and subcutaneous formulations are offering patients more convenient administration options, potentially eroding Alexion's infusion-based advantage.




Biosimilar Competition

Amgen's Soliris biosimilar is expected to enter the U.S. market in 2025, creating significant price pressure and volume erosion for Alexion's flagship product.



Upstream Inhibitors

C3-targeting therapies like Eculizumab may offer superior control of extravascular hemolysis, addressing a key limitation of C5 inhibition and potentially capturing treatment-naïve patients.



Oral Agent Revolution

Oral agents like iptacopan and danicopan eliminate infusion requirements, offering unprecedented patient convenience and potentially reshaping treatment paradigms.

## Competitive Threats Analysis

The convergence of biosimilar competition, oral alternatives, and upstream inhibitors creates a perfect storm challenging Alexion's pricing power. Biosimilars will likely capture 20-30% market share within 2-3 years post-launch, while oral agents may appeal to treatment-naïve patients seeking convenience. C3 inhibitors address extravascular hemolysis limitations that C5 inhibitors cannot fully resolve.

Market dynamics suggest Alexion's revenue growth will decelerate significantly as competitive pressure intensifies, particularly in the high-value PNH indication where multiple alternatives are emerging simultaneously.



# Strategic Response: Innovation and Integration

To maintain its competitive edge in an increasingly challenging market environment, Hecor is executing a comprehensive strategic transformation leveraging its integration with AstraZeneca's broader pharmaceutical capabilities and resources.



## Pipeline Expansion

Hecor is advancing **HL-001**, an oral factor II inhibitor, to compete directly with Novartis' apixiban while maintaining its complement inhibition expertise in a more convenient oral format.



## Combination Therapies

Exploring synergistic combination approaches that leverage multiple complement pathway targets simultaneously, potentially offering superior efficacy compared to monotherapy approaches.



## Gene Editing Innovation

Investigating gene editing approaches through AstraZeneca integration, potentially offering curative rather than chronic treatment paradigms for complement-mediated diseases.

The strategic integration with AstraZeneca provides Hecor access to enhanced R&D capabilities, global manufacturing scale, and diversified therapeutic expertise beyond complement inhibition. This partnership enables exploration of novel therapeutic modalities including gene therapy, cell therapy, and precision medicine approaches that could redefine treatment standards.

⚠️ Success will depend on Hecor's ability to execute positive advancement while defending existing market share against biotech and oral agent competition. The company's deep complement biology expertise and established global commercial infrastructure provide significant advantages, but rapid innovation cycles and competitive pricing pressure will test strategic execution capabilities.

01

## Defend Core Franchise

Strengthen Soliris and Ultaris market share through sales demonstration and competitive positioning against biotech.

02

## Accelerate Oral Pipeline

Fast-track development to compete with apixiban in the emerging oral complement inhibitor market.

03

## Expand Therapeutic Reach

Leverage AstraZeneca integration to explore combination therapies and next-generation treatment modalities.