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AstraZeneca's Eculizumab becomes first CDSCO-approved anti-complement medicine to manage two rare diseases

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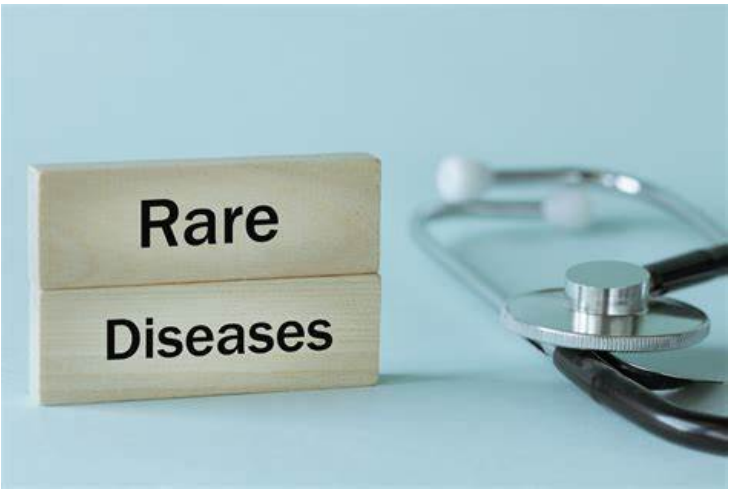
The approval boosts standard of care for rare disease patients in the country, marking a significant progress this upcoming Rare Disease Day 2025



AstraZeneca Pharma India has announced a Central Drugs Standard Control Organisation (CDSCO) permission to import, sale or distribute 'Eculizumab' concentrate solution for infusion 300 mg (10mg/ml).

Through this approval, Eculizumab becomes a first anti-complement medical therapy indicated for treating two rare conditions- Paroxysmal Nocturnal Hemoglobinuria (PNH) and atypical Hemolytic Uremic Syndrome (aHUS), both in children and adults.

PNH is a life-threatening disease characterised by thrombosis, end organ damage and impaired quality of life due to chronic haemolysis. Historical global data reveals poor survival rates, with 72% of patients dying within 25 years of diagnosis and without timely diagnosis and treatment options, patients face increased risks of complications such as thrombosis, renal impairment and premature mortality.

aHUS on the other hand, presents a grave unmet medical need in India, marked by devastating clinical manifestations, including thrombotic microangiopathy (TMA), acute renal failure, neurological complications, and multi-organ damage. These presentations often result in prolonged hospitalizations, increased healthcare costs, and substantial morbidity.

"With an ambition to be pioneers in science and lead in specialist disease areas, we are proud to bring this innovative therapy for rare disease patients in the country. This is a clear reflection of our purpose to expand access to novel therapies and transform patient outcomes across therapy areas where we believe we can make the most meaningful difference. This approval addresses long standing unmet patient need and offers hope to rare disease community in the country. Following this approval, we remain committed to collaborate with the ecosystem and advance access, putting patients first", said Dr Sanjeev Panchal, Country President & Managing Director AstraZeneca Pharma India Limited.