



## India Approves First Home-Grown Gene Therapy for Rare Disease

- **Regulatory Milestone:**  
The Central Drugs Standard Control Organisation (CDSCO) has granted approval for India's first indigenously developed gene therapy for Spinal Muscular Atrophy (SMA).
- **Development Journey:**  
A joint initiative between IIT-Kanpur and a domestic biotech start-up, the therapy underwent years of research, preclinical evaluation, and early-stage clinical trials to ensure safety and efficacy.
- **Affordability Factor:**  
Imported therapies for SMA currently cost ₹12–16 crore per patient. The Indian version is expected to be available below ₹1 crore, offering hope for thousands of affected families.
- **Significance:**  
SMA, a rare genetic disorder, weakens motor neurons, leading to progressive muscle wasting in children. The therapy works by delivering a functional SMN1 gene copy, aiming for long-term correction rather than temporary relief.
- **Future Outlook:**  
Experts believe this breakthrough could pave the way for gene therapies for thalassemia, hemophilia, and other genetic conditions in India.