Agreement 10: Rare Disease Enzyme Replacement Therapy

Parties: RareGene Therapeutics and Francophone Universal Health Consortium Country: France Disease Area: Gaucher Disease Type 1 Agreement Overview: This specialized agreement addresses the ultra-high cost of enzyme replacement therapy for Gaucher Disease Type 1, a rare lysosomal storage disorder affecting approximately 1 in 40,000 people. The contract covers approximately 165 patients across the French healthcare system and creates an innovative financing model that spreads costs over time while guaranteeing clinical effectiveness. The agreement includes special provisions for pediatric patients and long-term monitoring protocols.

Financial Structure:

- Annual therapy cost: €325,000 per patient for enzyme replacement (standard weight-based dosing)
- Annuity payment model: Payer distributes cost over 10 years at €32,500 annually per patient
- Performance guarantees:
    - 30% refund if no reduction in spleen volume within 6 months (measured by MRI volumetrics)
    - 25% refund if no improvement in hemoglobin levels within 3 months (minimum 1 g/dL increase)
    - Performance metrics stratified by disease severity at baseline
- Long-term outcome bonus: €50,000 per patient maintaining disease stability for 5+ years with no disease progression
- Genetic screening program: €1.2 million annual funding from manufacturer for at-risk populations
- Catastrophic coverage: 100% manufacturer coverage beyond €400,000 per patient annually
- Pediatric patient provision: Enhanced monitoring and specialized dosing with growth-adjusted pricing
- Alternative therapy comparison: Annual reassessment against emerging treatment options

Duration: 10 years with biennial reassessment and price adjustment mechanisms Special Provisions:

- Includes home infusion services to minimize hospital visits for stable patients
- International patient registry participation for data sharing and long-term outcomes tracking
- Specialized genetic counseling services for families
- Comprehensive multidisciplinary care team including hepatologists, hematologists, and geneticists
- Bone density monitoring and management of skeletal complications
- Annual patient-centered comprehensive assessment
- Transition program for pediatric patients reaching adulthood
- Pregnancy management protocol for female patients of childbearing age

- Emergency access provision for newly diagnosed patients
- Research partnership for next-generation therapies including gene therapy approaches