

Medical Discoveries & Breakthroughs in 2025: A Year of Precision, Hope, and Transformation

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2025 marked a remarkable chapter in medicine, characterized by rapid progress in personalized gene editing, safer pain management, cancer immunotherapy, and the integration of artificial intelligence into diagnostics and treatment. These breakthroughs not only addressed long-standing clinical challenges but also brought personalized and equitable healthcare closer to reality. From the world's first customized in vivo CRISPR therapy that transformed the life of a newborn with a rare metabolic disorder, to the approval of the first new class of non-opioid analgesic in decades, and compelling evidence linking the shingles vaccine to reduced dementia risk, this year demonstrated medicine's growing ability to intervene earlier, more precisely, and with fewer side effects. Advances in xenotransplantation, ultra-rapid genome sequencing, and AI-assisted surgical systems further expanded what is possible in organ replacement, rare disease diagnosis, and operative precision. Meanwhile, innovative approaches to turning "cold" tumors "hot" and regenerative skin grafts for severe genetic conditions offered new hope to patients with previously limited options. As we stand at the intersection of biology, technology, and global collaboration, these discoveries reinforce VejoVis Journal's commitment to supporting the multidisciplinary health workforce. By sharing and critically appraising such

advancements, we empower doctors, nurses, physician associates, researchers, and students to translate innovation into better patient care worldwide. Our core mission remains steadfast: "To look after the health workforce so they can look after you."

1. **Personalized In Vivo CRISPR Gene Editing (Baby KJ Case)**

The first fully customized CRISPR base-editing therapy was designed, tested, and delivered in just six months to a newborn with a rare, life-threatening urea cycle disorder (CPS1 deficiency). The treatment corrected the mutation in the liver, dramatically improved ammonia levels, allowed a more normal diet, and reduced the immediate need for a liver transplant. This landmark case opens the door to rapid, on-demand gene therapies for ultra-rare diseases.

2. **Non-Opioid Painkiller for Acute Surgical Pain (Suzetrigine / Journavx)**

The FDA approved the first new class of non-opioid analgesic in decades for moderate-to-severe acute pain following surgery. In large phase 3 trials, it provided effective pain relief without the risks of addiction or respiratory depression associated with opioids, marking a major step

in safer postoperative pain management.

3. **Shingles Vaccine Linked to Reduced Dementia Risk** Large real-world studies (including data from Wales) showed that the shingles vaccine reduced the risk of developing dementia by about 20% over seven years and may slow progression in those already affected. This supports the theory that herpes zoster virus contributes to dementia risk and offers a simple preventive strategy.
4. **Advances in Xenotransplantation (Pig Organs in Humans)** Patients received gene-edited pig kidneys (one surviving a record 271 days) and a partial pig liver. New techniques reduced rejection, and trials expanded under compassionate use, bringing xenotransplantation closer to solving the organ shortage crisis.
5. **AI-Powered Ultra-Fast Genome Sequencing** A new world record was set with whole-genome sequencing and analysis completed in under 4 hours (using Roche's SBX workflow). This dramatically speeds up diagnosis of rare genetic diseases and enables faster personalized treatments.
6. **New Targeted Treatments for Hard-to-Treat Cancers** Sevabertinib (an oral drug) showed promise for certain non-small cell lung cancers. Additional progress

included turning "cold" liver tumors "hot" with erythropoietin blockade plus immunotherapy (complete regression in mouse models) and new approaches for head and neck cancers.

7. **Progress Toward Male Birth Control Pill** A non-hormonal daily pill (YCT-529) successfully passed early human safety trials. It blocks sperm production reversibly by targeting a vitamin A metabolite receptor, offering a long-awaited new contraceptive option for men.
8. **Genetically Engineered Skin Grafts for Epidermolysis Bullosa ("Butterfly Disease")** Patient-derived engineered skin grafts healed chronic wounds and significantly reduced pain in clinical trials for this severe blistering skin condition.
9. **Expanded Robotic Surgery Systems and Telesurgery** New robotic platforms (including CMR Surgical's Versius) gained approvals for additional procedures. AI integration improved preoperative planning, real-time decision support, and training. Early robotic telesurgery trials advanced, allowing remote expert guidance.
10. **Smart Surgical Instruments and AI-Assisted Operating Rooms** Systems that record procedures internally,

analyze thousands of prior cases with AI, and provide real-time tissue/organ insights during surgery became more sophisticated, improving precision, reducing complications, and shortening learning curves for surgeons.