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4 RARE DISEASES DRIVING GLOBAL RESEARCH

Rare diseases are often at the center of **breakthrough therapies !**

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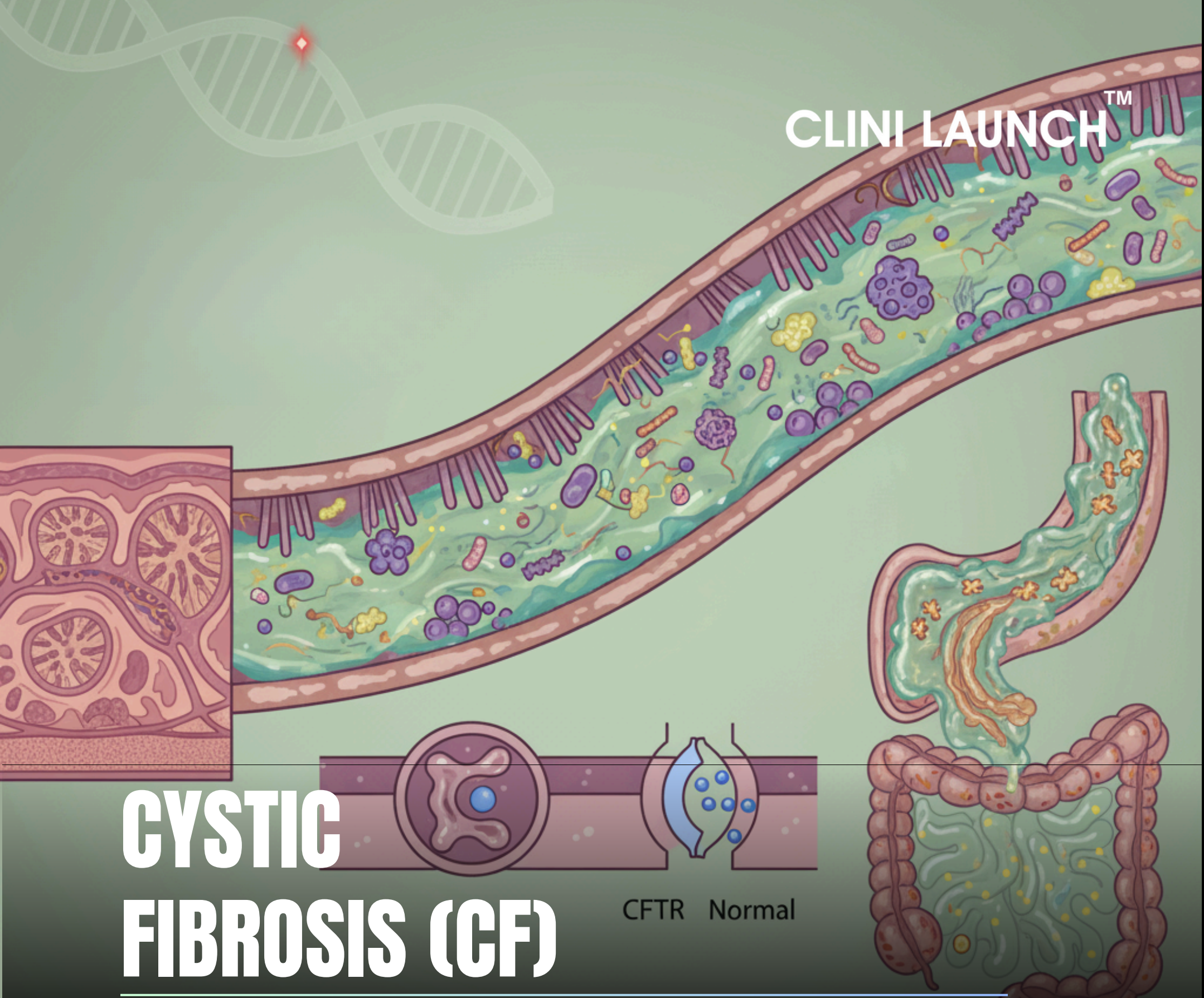

CYSTIC FIBROSIS (CF)

A genetic disorder caused by mutations in the CFTR gene.
It leads to thick mucus in the lungs and digestive tract, causing chronic infections and poor nutrient absorption.

Progress: Breakthrough drugs like CFTR modulators (e.g., Trikafta) have dramatically improved life expectancy and quality of life.

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DUCHENNE MUSCULAR DYSTROPHY (DMD)

A progressive muscle-wasting disease, usually diagnosed in boys before age 5.
It results from mutations in the dystrophin gene, leading to loss of mobility and shortened lifespan.

Progress: FDA approvals of exon-skipping drugs and ongoing gene therapy trials are showing promising mobility improvements.

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ALS & HUNTINGTON'S DISEASE

- **ALS (Lou Gehrig's Disease)** – A rare disorder where motor neurons die, causing muscle weakness, paralysis, and breathing difficulties.
- **Huntington's Disease** – A hereditary brain disorder causing uncontrolled movements, cognitive decline, and psychiatric issues.

Progress: Recent drug approvals like tofersen (ALS) and gene-silencing therapies (Huntington's) are giving cautious optimism.

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SICKLE CELL & HEMOPHILIA

- **Sickle Cell Disease (SCD) –** A blood disorder where red blood cells take a sickle shape, blocking blood flow and causing severe pain.
- **Hemophilia –** An inherited disorder where blood doesn't clot normally, leading to dangerous bleeding.

Progress: CRISPR-based gene editing (SCD) and long-acting clotting factors (Hemophilia) are changing treatment standards.

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WANT TO BUILD A CAREER WHERE EVERY STUDY MAKES A DIFFERENCE?

Save this post as a quick reference — and remember, at Clini Launch, you can turn knowledge like this into a career in Clinical Research.

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