

Disease pathways: A key to new drug discovery

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As recently as a decade ago, many scientists and physicians were unaware of the connections between seemingly disparate diseases. Osteoporosis and cancer, for example, were considered completely unrelated. But it turns out they sometimes share similarities at a molecular level.

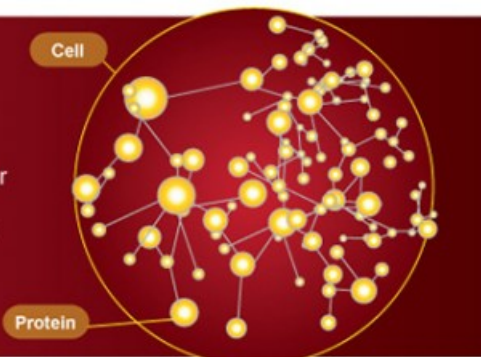
Disease Pathways

A Key to New Drug Discovery

Our metabolism, gene regulation and biological processes happen in molecular pathways. Disrupted pathways lead to disease. Scientists once thought all diseases were unique. New research shows that some diseases — cancer, psoriasis and arthritis, for instance — actually share molecular pathways and can be treated similarly. This breakthrough has already produced treatments that are more effective — and discovered more quickly — than any before.

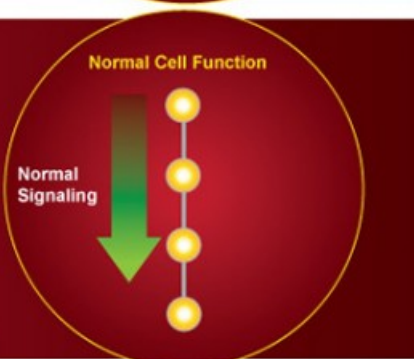
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The Novartis Institutes for BioMedical Research are studying complex protein networks known as molecular signaling pathways inside human cells. These proteins are generated from our human DNA.



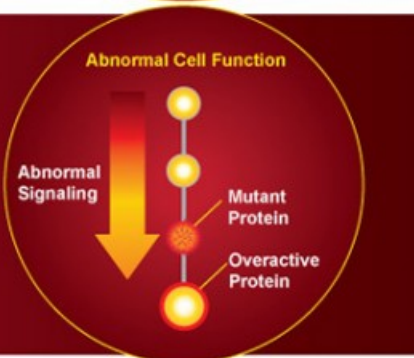
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Molecular pathways are highly controlled and interconnected signal-relay systems, similar to telecommunication networks. They are responsible for normal cell function.



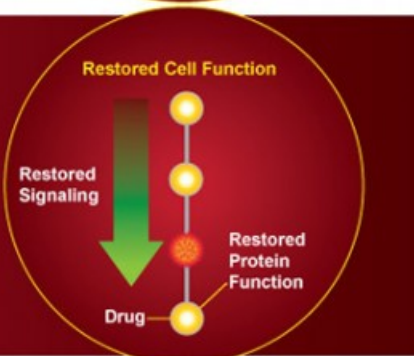
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When a protein in a pathway does not function properly, the result can be abnormal signaling and disease. Excessive cell growth in cancer is often the consequence of mutant proteins and a resulting loss of pathway control.



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Researchers discover potential drug targets by identifying which protein to treat in a pathway. By determining the right targets, Novartis hopes to create medicines that will restore pathway function and treat a variety of diseases.



Using new technologies, researchers have developed a deeper understanding of how cells function, changing conceptions of disease. They're mapping the complex network of molecular pathways that control a cell's activities. Each pathway includes many players that interact. A series of signals, from one molecule to another,

triggers particular actions — the production of a protein, for example, or cell division. Defects in a pathway can cause disease.

The latest maps of molecular interactions reveal connections between diseases that seem very different. A molecule linked to bone loss, for example, appears in the same pathway as a molecule associated with certain types of cancer. In fact, a relatively small number of core pathways play a fundamental role in development — and in disease. Researchers at the [Novartis Institutes for BioMedical Research \(NIBR\)](#) focus on these key pathways, searching for ways to maintain or restore their function to treat a variety of conditions.

Identifying the location of the pathway disruption is a first step toward developing new medicines. Then scientists need to figure out how to fix the problem. This approach has already produced more effective treatments for diseases such as multiple sclerosis and chronic myelogenous leukemia, but there are still many more opportunities to explore.

NIBR researchers continue to work to unravel how complex pathways are structured in order to find ways to intervene in disease. Although the course of each pathway is intricate and requires insights from multiple disciplines, a complete understanding of pathways and the roles they play within cells and the human body could help deliver profound results that could provide important therapies for some of the world's most deadly and difficult-to-treat diseases.

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