Modular Product Engineering

Remedium's Approach to Product Development

REMEDIUM

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The Covid pandemic has once again critically highlighted how important pharma R&D timelines are to saving lives and reducing human suffering. In addition to the immediate humanitarian need of new medicines, short development timelines also increase affordability and access to treatment. In 2015, an analysis conducted by Northwestern University reported that a typical drug approval timeline takes 15 years, and only one in 5,000 to 10,000 research compounds will make it to market following rigorous preclinical and clinical evaluation. Gene therapy is changing this paradigm, and the gene-based Covid vaccines have validated a new principle – using modular product engineering can save time, money, and bring more effective and safe medicines to market quickly and efficiently.

The development of small molecules relied on systematic chemical modification of an active ingredient to improve safety and efficacy, a process of 'guess and check'. With the advent of liquid handling automation, thousands of small molecule compounds could be synthesized and then screened on standardized cellular bioassays. Automation improved throughput but did little to improve the overall probability of success. In silico / computer modeling of the fit and affinity between small molecules and their targets was then used to predict binding strength and specificity; however, the complexity of biological systems, and the number of possible interactions limited the effectiveness of such modeling for synthetic drugs. With the advent of monoclonal antibodies and recombinant proteins, we were finally able to develop therapeutics which had clearly defined, specific biological targets. Furthermore, these therapies had predictable pharmacokinetic and pharmacodynamic profiles as they were shuttled, distributed, and resorbed via natural pathways that our cells normally utilize to transport and clean-up similar biomolecules.

While antibodies were a breakthrough, they are generally only able to target structures on the outside of cells, at the same time, the majority of activity and therapeutic potential lays intracellularly. This is where the final piece of the puzzle – nucleic acid-based therapeutics, ranging from oligonucleotides, messenger RNA, and traditional gene therapy plays a central role. Nucleic acids and derivatives, delivered to the inside of the cell by viral or non-viral vectors were finally able to provide to biomedical engineers and pharmaceutical scientists the tools to target nearly any intracellular activity in a highly specific manner. Drug-development has transcended basic science and entered the realm of engineering.

Small Molecules

10,000-15,000 candidates
15+ year developments
Uncertain safety and efficacy

Gene Therapies

Known biological product
Specific activity of expressed protein
Natural process for bioresorption

Remedium's approach to product development utilizes modular application of clinically characterized biological constructs to treat well established disease pathophysiology. Our scientists and engineers methodically analyze and combine proven promoter sequences, nuclear transport signals, post-translational response elements, delivery vectors, and formulations, tailoring them to replace, repair, or augment known molecular deficiencies that result in disease. By engineering proven scientific concepts into novel therapeutic constructs, we are able to significantly reduce development timelines and de-risk clinical trials. Furthermore, when selecting conditions, Remedium targets pathologies with well characterized molecular mechanisms, ideally, where the disease has already been successfully approached with intermittent protein-based treatment. This methodology allows for efficient development of a more efficacious and durable solution, ideally suited to gene therapy, while reducing the uncertainty of preclinical and clinical R&D.

Modular	=	Proven	+	Novel	+	Targeting
Product Engineering		Technological Modalities		Engineering Combination		Characterized Pathology

Use of proven technological modalities, in novel ways, targeting well characterized disease pathology are the three principles that form the foundation of Remedium's modular product engineering, an efficient way to bring affordable medicines to market quickly and effectively.

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Remedium Bio is a Boston area gene therapy company focused on the development of highly innovative treatments for a broad range of diseases. The Remedium approach utilizes proven scientific fundamentals, in a novel way, to treat the most debilitating conditions. Starting with well-characterized pathophysiological principles, our scientists modularly apply proven technologies to quickly advance curative treatments through the R&D cycle. Today, our leading candidates include gene therapy treatments in the fields of Endocrinology, Cardiology, Hematology, and Rheumatology.