"Ribosome Editing for Rare Skin Disease and Elastin Regeneration"

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Abstract
The ribosome, a ribonucleoprotein complex central to protein synthesis, is frequently dysregulated in cancer due to increased biogenesis and mutations in ribosomal proteins, promoting tumor cell proliferation and altered cellular metabolism [1]. Recent evidence indicates that disruptions in ribosome production—whether inherited or acquired—are linked not only to tumorigenesis but also to various rare genetic disorders, termed ribosomopathies [2]. Targeting ribosome biogenesis and function has thus emerged as a promising anti-cancer strategy, with several agents being developed to modulate this essential machinery. Notably, selective modulation of specific ribosomal proteins can restore full-length protein synthesis in genetic diseases like severe junctional epidermolysis bullosa, as demonstrated by the use of artesunate and atazanavir—ligands for ribosomal protein L35/uL29—which significantly increased levels of functional LAMB3 protein in cell models [3]. Additionally, the RiboScreenTM technology enables the identification of both ribosomal protein targets and small-molecule modulators, leading to enhanced synthesis of proteins such as tropoelastin, with therapeutic potential for skin and cardiovascular rejuvenation [4]. Together, these approaches highlight the translational potential of ribosome-targeted therapies in oncology, rare diseases, and regenerative medicine, despite ongoing challenges regarding selectivity, toxicity, and clinical application.

References

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