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Novel Pharmacological Treatment of Scleroderma

This invention relates to a novel pharmacological treatment of scleroderma comprising the administration of miR-29.

Keywords Scleroderma, miR-29

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Background Scleroderma is a chronic autoimmune disease characterized by a fibrosis of the skin and internal organs. The high morbidity and mortality is the consequence of the failure of affected organs such as the lungs. In the United States alone approximately 300.000 people are affected. To date there is no therapy available to slow or prevent the disease progression. Therefore, there is a substantial need for a specific anti-fibrotic therapy. miR-29 is a small non-coding micro RNA that is involved in regulating gene expression.

Invention This invention relates to a novel pharmacological treatment of scleroderma (SSc) comprising the administration of miR-29. miR-29 was found to be strongly downregulated in SSc fibroblasts as well as skin biopsies from SSc patients compared to healthy controls. Furthermore the enforced expression of mi-R29 in SSc skin fibroblasts resulted in downregulation of collagen on mRNA and on protein level. These experiments provide robust evidence that the miR-29 family negatively regulates major pathogenic pathways in SSc.

Fields of Use Based on this invention miR-29 is a valuable candidate for the development of a pharmaceutical composition for the treatment of scleroderma.

Patent Status Patent application filed.

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