At the forefront of the introduction of gene therapy in hemophilia A and B: Design and implementation of an innovative software platform („smart medication Gene“) for gene therapy

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Introduction

In the treatment of rare diseases, especially in hemophilia A and B, gene therapies will strongly increase in importance in the coming years. The complex process of qualification of treatment centers, preparation of patients for therapy, implementation of treatment and subsequent follow-up, including registry reporting over many years can only be implemented with suitable software tools.

Method

The aim is to record all gene therapy treatment data in a structured manner in a single software tool and to document it in a specialist patient file. Furthermore, in addition to the documentation requirements of physicians and treatment centers (HCPs, CCCs), such as the patient's suitability for gene therapy, the requirements of the regulatory authorities (EMA, GBA) are also to be recorded. The software tool named „Gene“ also supports the collaboration of centers according to the so-called “hub & spoke model”. In the hub & spoke model, there is a formalized division of tasks between the centers, i.e. the various necessary activities are assigned to either a „hub“ or a „spoke“ center, thus organizing and supporting collaboration between the centers. Core elements and functionality of Gene are shown in the figure.

Results

Gene covers all HCP and regulatory (EMA, GBA) requirements related to the recently approved gene therapy in hemophilia A. Gene is currently being validated in selected hemophilia centers in Germany (both dosing and referring centers).

Conclusion

The complex treatment process in gene therapy with the high demands on collaboration between treatment centers, documentation and compliance requires an integrated software tool. The focus should be on a targeted collaboration between the service providers to ensure optimal preparation, therapy implementation and follow-up of patients in gene therapy.

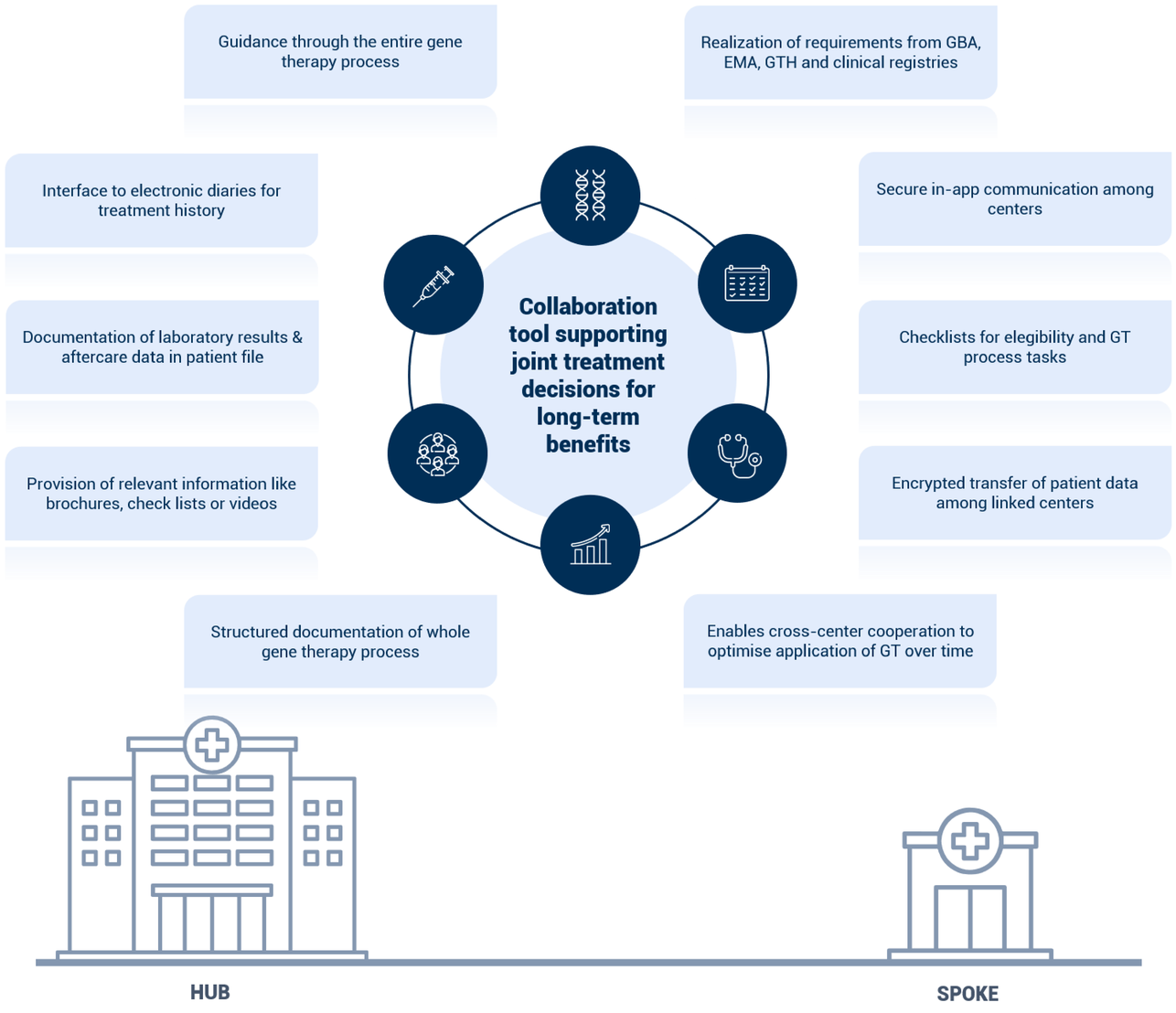

Figure: Hub & Spoke requirements implemented in GENE