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Title: Gene Therapy – Current Status and Possibilities in Clinical Medicine

Agencies SMM, The Norwegian Center for Health Technology Assessment
SINTEF Unimed, Forskningsveien 1, Postboks 124 Blindern, NO-0314 Oslo, Norway
Tel: +47 22 06 78 08 Fax: +47 22 06 79 79

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In this early warning, an expert group has identified 401 gene therapy protocols focusing mainly on their current status in clinical medicine and the promising results from preclinical research. The expert group assessed the possibilities for gene therapy in the future, in particular, diseases that may be relevant for this type of intervention.

Between three and four thousand patients worldwide have been treated with different gene therapy strategies in more than 400 clinical studies. Gene therapy today is dominated by preclinical and clinical research. Of several thousand published articles on gene therapy, only approximately 80 articles describe results from clinical trials. Most of these studies are phase I or II with few patients in each study. Only three of the protocols represent phase III studies. None of the existing international databases of gene therapy protocols are complete. SMM has constructed a database of the identified protocols. Apart from the use of soluble antisens oligonucleotides against cytomegalovirus (CMV), which has been approved in the United States for use in eye infections among AIDS patients, gene therapy is currently not an established treatment modality for any disease. Gene therapy has been tested in particular for different genetic diseases, including both inherited diseases, resulting from mutations in a single gene and diseases where mutations in several genes can contribute to disease development, such as cancer. In addition, gene therapy can also be implemented in certain diseases to add a drug or an active component. While most gene therapy studies have been performed in the treatment of cancer, some have targeted viral infections (HIV in particular, but also others) and monogenic diseases where the gene is characterized and cloned. Recently, several studies concerning cardiovascular diseases have been initiated, and trials have also commenced in autoimmune diseases and certain neurological diseases such as amyotrophic lateral sclerosis (ALS). The report will be available in English in April/May. For more information: www.sintef.no/smm

SMM Conclusions

- Promising results have already been observed in early cancer clinical trials and most recently in certain forms of cardiovascular diseases.
- Gene therapy has developed to a point where it is now important to build up national competence in the field.
- Somatic gene therapy is ethically sound. This does not represent a new ethical principle. Gene therapy must be reserved for treatment of serious diseases.
- Both children and adults should be evaluated for gene therapy research.
- Gene therapy in utero should not be performed until more knowledge is available.
- Germline gene therapy or gene manipulation of embryos or germ cells should be forbidden in accordance with international consensus and Norwegian law.
- Genetic enhancement should not be allowed under any circumstances.
- Research on the ethical issues related to gene therapy should be supported.

More translational research – between basic and clinical research will prevent establishment of new and exciting methods before their clinical effect has been demonstrated.

Written by Dr. Anita Lyngstadaas, SMM