GENETIC ENGINEERING AS A FUTURE HOPE IN PEDIATRICS

Essay

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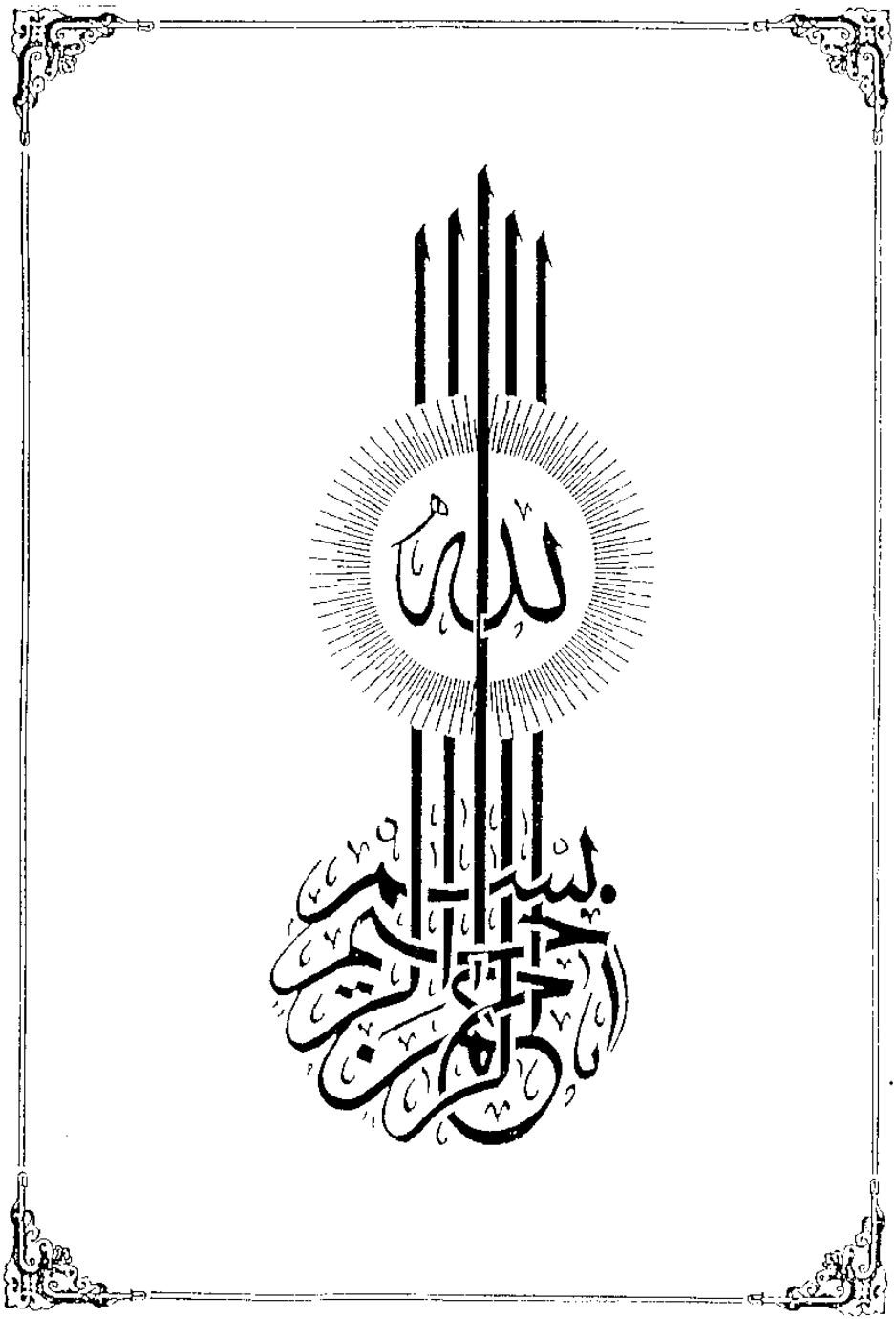
بسم الله الرحمن الرحيم

﴿قَالُوا سُبْحَانَكَ لَا عِلْمَ لَنَا إِلَّا مَا عَلَّمْتَنَا إِنَّكَ أَنْتَ الْعَلِيمُ الْحَكِيمُ﴾

«صدق الله العظيم»
(سورة البقرة آيه رقم (٣٢)



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Nagwa M. Zaki.

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LIST OF ABBREVIATIONS

AAV	Adeno associated virus
AAT	Alpha-1 antitrypsin
A	Adenine
ADA	Adenosine deaminase
Ado	Adenosine
AIDS	Acquired immunodeficiency syndrome
ASO	Allele specific oligonucleotide
BMD	Becker muscular dystrophy
BMP	Bone marrow transplantation
bp	Base pair
C	Cytosine
cAMP	Cyclic adenosine monophosphate
cDNA	Complementary DNA
CFTR	Cystic fibrosis transmembrane conductance regulator
CF	Cystic fibrosis
CNS	Central nervous system
dAdo	Deoxyadenosine
DMD	Duchenne muscular dystrophy
DNA	Deoxyribonucleic acid
FH	Familial hypercholesterolemia
FIH	Fluorescence in situ hybridization
G	Guanosine
Gc	Glucocerebrosidase
HCT	Hepatocellular transplantation
HIV-1	Human immunodeficinecy virus-1
HPRT	Hypoxanthine phosphoribosyl transferase
IL-2	Interleukin-2
kb	Kilo base
LCR	Ligase chain reaction
LDLR	Low density lipoprotein receptors

mRNA	Messenger RNA
OTC	Ornithine transcarbamylase
PCR	Polymerase chain reaction
rec A	Recombinase A
RFLP	Restriction fragment length polymorphism
RNA	Ribonucleic acid
SCID	Severe combined immunodeficiency
T	Thymine
TIL	Tumor infilterating lymphocytes
TNF	Tumor necrosis factor
U	Uracil
VIIIc	Antihemophilic factor
VNTR	Variable number of tandem repeats
VWD	Von willebrand disease
VWF	Von willebrand factor

INTRODUCTION AND AIM OF THE ESSAY

INTRODUCTION

Genetic engineering is the application of recombinant DNA technology (Zasloff, 1992).

In broad terms, applications of recombinant DNA technology can be divided into four areas: biomedical, basicbiological, agricultural, and industrial. Biomedical applications include the elucidation of the cellular and molecular bases of a broad spectrum of diseases, as well as in clinical medicine where both diagnostic and therapeutic applications are being pursued (Kappy et al., 1983).

Recent advances in recombinant DNA technology have led to an increase in our understanding of the molecular basis of many genetic diseases. Approximately 3500 different human genetic diseases are known, and as the genes responsible for these diseases are identified and cloned, many advances in treatment will be made. These advances have already been translated into improved methods for the prenatal diagnosis of many diseases and the use of recombinant gene products in treatment regimes (Anderson 1984, Kantoff et al., 1988, Williams 1988, Friedmann, 1989).

Recombinant DNA procedures have now been applied for the identification of molecular defects in man that account for heritable diseases, somatic mutations associated with neoplasia, and acquired infectious diseases. Thus recombinant DNA technology has rapidly expanded our ability to diagnose disease. There can be no doubt that DNA diagnosis has already made substantial contributions to the diagnosis of disorders such as sickle cell anaemia, thalassemia, Duchenne muscular dystrophy and cystic fibrosis (Caskey, 1987).

Therapy of genetic diseases may be attempted at three different levels in the evolution of the disease process. At the first level after clinical manifestations have appeared, treatment is symptomatic. At the second level, mid way between the origin of the disease and the appearance of clinical manifestations, therapy consists of adiminstration of a normal gene product such as insulin in diabetes and factor VIII in hemophilia. At the third level, the origin of the disease, methods involve correcting the gene defect and are currently under investigation (Karp, 1980).

The DNA technology has already resulted in the synthesis in microorganisms of a number of useful proteins such as vaccines, insulin and interferon (Baxter, 1983).

However, with further developments in recombinant DNA technology it will soon be possible to correct the genetic defects themselves in affected individuals, through the use of somatic gene therapy techniques in which the gene is only introduced into the somatic cells of the patient and not into the germ line. Therefore, the gene can only be expressed in those cells into which it was introduced and their progeny cells, but it can not be passed on to subsequent generations of children (Kinnon et al., 1990).

AIM OF THE ESSAY

This essay will be done to study the impact of genetic engineering and DNA technology on the future of pediatric problems.