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Are we near the cure of genetic diseases and possibly cancers, in the advanced CRISPAR era??

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A frozen bag of genetically engineered blood or a bag of cells can change the life of persons forever; this is the new era of precision gene medicine. Many debilitating diseases can be completely cured with some financial toxicity, but in front of a lifetime suffering this financial toxicity is bearable and manageable.

Sickle cells disease (SCD), a blood disorder which damages multiple organs and excruciating pains leads to multiple morbidities. SCD occurs because of the defect in HBB gene, single mutation in this gene leads to formation of haemoglobin S, which leads to sickling, sticky and crescent-shaped red blood cells in slight hypoxic conditions. Leading to strokes, heart attacks and other critical organs damages in multiple ways in multiple times over period of time. This increases morbidity and early mortality. As this is disease is recessively heritable from parents and high degrees of penetrance in off springs. This disease thought to have originated in Africa and have protective effects against endemic malaria.

With the CRISPAR gene editable technique this single gene mutation can be edited timely in a patient's blood stem cells outside the body with advanced engineering and reengineering techniques and after a quality check, this modified blood can be retrans fussed to form sickle less blood cells.

Techniques and technologies are on-going to improve this simple yet advanced technique to cure similar diseases more effectively and efficiently. CRISPAR enable scientists to rewrite, delete or modify etc on target gene DNA and pave the way for cure and eradication of host of morbid genetic disease like Down's syndrome, cystic fibrosis, dyslexia, hereditary

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blindness, SCD and possibly cancers also. CRISPAR techniques can also be used in embryo in specific genes to customise baby for our genetic and phenotype interests. Till now the only risky treatment for few genetic diseases including SCD was bone marrow transplantation with high degrees morbidity and mortality.

CRISPAR allows altering or modifying any organisms' genetic sequence at our will for the benefit of humanity within the ethical constraints. CRISPAR based techniques can be united for any simple and complex diseases, now CRISPAR techniques used to get fatter goats with longer hair, yield more meat, wool, protect bacteria in making healthy cheese and yogurts, removing caffeine gene from coffee plants, breed micro pigs as pets as well as for Clinical Trials, for meat, disease resistant pet animals and possibly humans, to date gene editing on human embryos is selectively prohibited. Gene editing can be used in congenital deafness and blindness where ethical constraints are there to utilise in human embryos, it can be selectively done in *invitro* and in *vivo* and *invitro* gene editing can be done selectively to decrease the human sufferings without breaking ethical constraints.

CRISPRs rational goal is to fix, cure and or reverse genetic mutations that is or are leading to illnesses or otherwise short-change of lives. CRISPR offers a far more accessible promise, the ability to vanish and cure hereditary diseases and syndromes and sufferings. CRISPR can also leads to off-target consequences and side effects but CRISPR itself has answers this consequence and off target side effects. One good example is Fragile Xsyndrome, here genetic cause leads to severe autism, increased sensitivity to light, sound and repetitive obsessive-compulsive diseases and in Duchene muscular dystrophy affecting muscles in boys and is very debilitating since from birth. CRISPAR has permanent solutions to symptoms without affecting the basic gene.
Duchene muscular dystrophy a severe degenerative disease from a mutation in the largest gene known in humans, the dystrophin gene. Boys with these diseases are born healthy but because they lack an important protein that acts as a shock absorber for all affected muscles, these muscle fibers start breaking down by preschool age, by middle school unable to walk and by adulthood need ventilation for breathing. CRISPAR has answers to this disease like much other disease if done at the earliest with no unnecessary delay.

So with available CRISPR and NGS, it is theoretically and hopefully practical in curing many genetic diseases before the birth or after birth and so on.

As we now know that all cancers are caused by changes in nuclear genetic material in the form germline and somatic mutations, there has been continuous search for curing these diseases by surgery, chemotherapy, radiotherapy in the last century. However, we have achieved very little in the path of curing most cancers. With advances in targeted therapy, immunotherapy and gene therapy, these therapies mainly based on the genetic alterations on the incitation, progression and metastasis of cancers, now there is more hope of curing cancers than the time a few decades ago. With better understanding of genetics and the complexity of cancers and the available techniques and technologies like Next Generation Sequencing (NGS) of tumour cells, immune cells and supporting cells, genetic material corrections can be done using the advanced CRISPR technology.

By carefully editing the required targeted genes in immune cell either in *vivo* or *in vitro* can better prepared to attack cancer cells, by knowing the correct genetic alterations in the cancer cells we can use appropriate targeted drugs against the cancer cells, immunotherapy can be used to alter the interaction between cancer cells and immune cells and finally effectively kill the cancer cells by changing the microenvironment of tumours and immune cells. With CRISPR technology any cell DNA

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can be edited using guide RNA and DNA cutting enzyme called Cas9. Scientists design the guide RNA to mirror the DNA of the gene to be edited called targeted DNA, by editing genes this way, genes can activate or deactivated according to the function we need to kill or eliminate cancer cells precisely without affecting the normal cells.

CRISPR is simple, easy to use, customisable, scalable and fast, can create different biological models using bioinformatics, artificial intelligence and next generation sequencing. CRISPR can be used as diagnostic as well as development advanced therapeutics. With a lot of refinements and advancements in this CRISPR, the days are nearer of dreaming cure cancers and other debilitating acute and chronic diseases. Gene therapies, CART cell-like treatments, etc for different types of cancers are well-come news of hope due to the advancements in CRISPR gene editing technology [1-6].

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