ALTERNATIVE METHOD FOR THE TREATMENT OF SCID

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Annotation: Severe combined immunodeficiency (SCID), also known as "bubble boy disease," is a rare hereditary pathology wherein patients lack an immune system. The most common form is X-linked SCID, caused by genetic mutations of IL2RG, which is needed for the development of immune cells - T and B lymphocytes.

Key words: SCID, bubble boy disease, T and B lymphocytes.

If a child is diagnosed and treated within the first few months of life before the child has a serious infection, then the long-term survival rate is more than 90%. With early treatment, most children with SCID should be able to develop their own working immune system. The best course of treatment for a child with SCID depends on several factors including the type of SCID, the child's health, and doctor recommendations. Most infants with SCID are treated with HSCT, or bone marrow transplant, which results in a new immune system that is able to fight infection. In HSCT, doctors take healthy blood-forming cells that can develop into a healthy immune system from a donor and put them into a child. The donor cells provide the child with an immune system. Another less common but promising treatment option is gene therapy, which is currently in clinical trials. In gene therapy, doctors extract a child's defective blood-forming cells, correct the defect, and put the corrected cells back into the child. The repaired cells provide the child with a working immune system. A third treatment, enzyme replacement therapy, can be used for children and adults with ADA-SCID. In enzyme replacement therapy, the missing enzyme is regularly injected into the person with SCID to boost the ADA enzyme. The results are temporary and do not permanently repair the immune system. Eventually a person with ADA-SCID will require either HSCT or gene therapy for long-term results. Prior to these treatments, a child with SCID will begin the treatment process by taking antibiotics, antivirals and antifungals to ward off infection. The child will also receive immunoglobulin therapy, or Ig, an infusion of antibodies designed to boost the child's immune system. The Ig is obtained through human plasma donors

The aim of work was to analyze out the main ways of treatment of SCID. Nowadays, SCIDs can be successfully treated by allogeneic HSCT (hematopoietic stem cell therapy) and bone marrow transplant (BMT). The Alternative option that could be taken into account is gene therapy.

For gene therapy, we gather a patient's own blood cells. For this, the patient is given injections of medicines that make the blood stem cells leave the bone marrow and enter the bloodstream. The stem cells are then collected by a process called apheresis, in which blood is drawn from the body and processed, after pulling out the blood stem cells, the remaining is returned to the patient. Physicians then modify the collected cells by inserting new genetic material or editing genes so that the cells can better produce hemoglobin. The patient then receives chemotherapy to clear out the existing bone marrow and "make space" for the modified cells. The modified cells are injected into the patient like a blood transfusion. Within 14 to 28 days after the procedure, the new stem cells should begin to grow in the patient's body and start to produce new blood cells.

Unlike allogeneic transplants from alternative donors, survival after gene therapy is excellent. In comparison with BMT, gene therapy presents a better safety profile and engraftment of multilineage transduced stem cells, due to the use of non-myeloablative preconditioning. While BMT requires finding a suitable donor, gene therapy uses a patient's own cells. By using a patient's own cells, there is no risk of graft-versus-host disease. Following BMT, patients may need immunosuppressive drugs, whereas these are not needed with gene therapy because patients are receiving their own modified cells.

Summary: SCID is one of the most serious forms of immunodeficiency diseases. Without successful treatment, the individual is at constant risk for a severe or fatal infection. One of the most successful methods of SCID treatment today is GENE THERAPY with the advantages of reducing the risk of transplant rejection and patients' better survival.