MANAGEMENT OF HAEMOPHILIA IN MALTA

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Haemophilia is a blood clotting disorder in which one of the essential clotting factors is deficient, Haemophilia A, or Classical Haemophilia is the most common form and is due to the deficiency of Factor VIII. Haemophilia B, or Christmas Disease is due to the deficiency of Factor IX. Haemophilia is hereditary. It affets males almost exclusively and is passed on through unaffected females who carry the defective gene. However, between one quarter to one third of all occurrences appear in families with no previous history of the disorder. Once haemophilia appears it is then passed on in the same genetic pattern. Approximately one in 15,000 males has the disorder. In Malta there are 23 males who have haemophilia. It is a life long condition. The development of clotting factor concentrates has meant that it can be managed effectively. Bleeding is mostly internal. The deficiency in clotting episodes usually into the muscle joints. These bleeding episodes may occur spontaneously (apparently without cause, or as a result of trauma). The bleeding is stopped by the intravenous infusion of appropriate clotting factor. If internal bleding is not quickly stopped with treatment it may result in pain and swelling. Over a period of time bleeding into joints and muscles can cause permanent damage, such as arthritis in the joint and chronic pain (Hilgartner et al).

Methodology

Study 1: Stability Work

Experimental work was carried out on the Activity of Factor VIII during viral inactivation with chemical detergent Triton x100. Blood was obtained from a healthy donor and this was treated with the chemical detergent. The concentrations used were: 0.001%, 0.005%; 0.01%; 0.05% and 0.1% together with 1ml of plasma, 0.1ml of factor deficient plasma 0.1ml KCC reagent and 0.1ml Calcium Chloride. The method of analysis used is known as the Kaolin Cephalin Clotting Time (KCCT) (Biggs).

Study 2: Survey

A limited survey was carried out based on personal interviews to the physicians treating the patients and to the Chairman of the Haemophilia Society. The reason of the study was to determine:

(a) The number of patients and the most common complications from receiving treatment.
(b) The most common bleeds.

(c) The cost of treatment.

Results

Study 1: Stability Work

This is a method used as part of the purification process during viral inactivation process. From the results obtained (Table 1) one can identify that the chemical detergent used in the inactivation of viruses had considerable affect on the activity of the Factor VIII. From the concentrations of Triton x100 used only the minimum concentration showed a low activity loss.

Table 1:

Substance	Av Time/sec	Activity
Calibration plasma	55.5	100.0%
Plasma + 0.001% Triton	58.3	89.5%
Plasma + 0.005% Triton	66.6	52.5%
Plasma + 0.01% Triton	70.3	27.5%
Plasma + 0.05% Triton	75.5	12.5%
Plasma + 0.1% Triton	79.9	5.0%

Study 2: Survey

(a) By the end of 1991, 23 patients were identified to be suffering from haemophilia of which all were males and 80% of these were due to a family hereditary disorder. Of these 23 patients, 78% (18) were diagnosed to be suffering from haemophilia A and 22% (5) from Haemophilia B. The degree of haemophilia was found to be 43% (10) were severe 26% (6) moderate, 13% (3) mild and 18% (4) not known. Complications treatment were, 34% (8) were diagnosed to be suffering from HIV, 8% (2) developed inhibitors to Factor VIII therapy, 4% (1) had a case of thrombosis and no cases of Hepatitis were yet reported. Only 65% (15) use Home Therapy while 35% (8) call physicians to treat bleeds.

(b) On clinical studies done the reason for most common bleeds were as shown in Table 2.

(c) The cost of management plan was also carried out for the treatment of such patients (Table 3).

Table 2:

Nature of Injury	Patient % (n=23)
Spontaneous bleeds	100%
Haemarthrosis	91%
Haematuria	17%
Gum Bleeding	82%
Epistaxis	86%
Intracranial	4%
Joint	65%
Muscle	39%

Table 3:

Number of patients	33
Number of units per patient	13,000 I.U.
Total number of units for all patients	
..... 1991	1,725,000 I.U.
..... 1992	1,600,000 I.U.
Cost of Therapy	
500 I.U. (1 Box of Factor VIII Concentrate)	58.31 Malta pounds
Cost per patient annually	8,749.47 Malta pounds
Total cost for all patients	201,238.00 Malta pounds

Management of Haemophilia

It is now widely acknowledged that haemophilia A and B should receive early treatment for every episode of bleeding however trivial. In some cases patients themselves have assessed the degrees of severity of bleeds and immediately administered infusions themselves or called the physician home for infusion of Faction VIII. A few years back Maltese Haemophiliacs were treated with cryoprecipitate which is derived from plasma, obtained from blood donors. This was a risk as people became aware that infectious viral infections could be transmitted. now Factor VIII is commercially being produced from Maltese plasma donors thus increasing safety in the treatment of haemophiliacs.

Importance was given to various topics such as Home Therapy, Patient Counselling, Treatment, Management and Improvement to the haemophiliacs in Malta.

Conclusion

Improvement of haemophilia care around the world by the century depends on persistence in, and support for possible means of cure by gene therapy[3]. The studies showed that adequate importance should be giving during viral inactivation to have highly effective Factor VIII concentrate to avoid complications in treatment. Factor VIII therapy has improved the treatment of haemophiliac patients, during bleeding episodes as this reduces the formation of swelling andpain.

The following recommendations should be adopted to have better management of haemophilia in Malta:

1. Supervised Home Therapy. A guide is being presented to present a step-by-step method of how a patient can with the help of an experienced physician or pharmacist receive therapy on his own whenever he requires to treat any bleeds.

2. Safe adequate supply of factor concentrate achieved through blood transfusion.

3. Maintenance of haemophiliacs' good physical condition by normal activities.

4. A good protocol for replacement therapy.

5. Specialised haemophilia centres, were patients should be diagnosed and treated.

6. Introduce patient counselling services to increase knowledge to the patient, relatives, friends, school teachers, work-mates and others. A guide is also presented to give a general outlook about haemophilia and helps the patient to understand himself better.

References

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