

# a project share story



PROJECT  
SHARE

It's time to give back

Imran Zia

## No Distance Too Far

This is the story of five brothers, all with severe hemophilia A: Izhar, Muneeb, Faheem, Daud, and Fazal. They live in a small village called Tank, in Pakistan. Tank is about 310 miles from where I live, in Lahore, capital of the Sindh province. When I visited the brothers, it took me about eight hours to travel there by car.

All the brothers' major joints are damaged due to untreated bleeding, from the lack of factor concentrates and cryoprecipitate. When they bleed, they must travel several hours to get plasma from other cities. Our organization, Hemophilia Care of Pakistan, provides factor to the brothers in their homes for free, whenever we can find a donation.

We celebrated World Hemophilia

Day on April 17 this year with these five brothers to raise awareness of the importance of physical therapy and hydrotherapy to reduce bleeding episodes. One brother, age 28, cut his leg and had a bone infection

in his left knee joint. His father, a retired schoolteacher, spent his entire pension for four surgeries on his son's knees—\$10,000 to purchase factor and to pay hospital expenses. A lifetime of savings!

Right now, his other son has a hematoma in his brain. We provided him with 40,000 IU of factor over ten days. Surgery is expected at any time, and he needs 40,000 IU more.\*☺



Above: A lifetime of savings to save brothers Izhar, Muneeb, Faheem, Daud, and Fazal, with Imran Zia (second from right). Right: Aquatherapy when facilities are lacking.



Imran Zia has hemophilia, and is president of Hemophilia Care of Pakistan, an organization that provides medical care and social advocacy to patients with hemophilia in Pakistan.

\*Project SHARE was able to donate factor for this patient's surgery.

### New Factor... from cover

## Product Types

Products are bought and sold on a *market*. In the bleeding disorder market, “products” means factor. To understand what new factor products are coming to market, you first need to know what's currently available. Factor concentrates are classified in several ways. The most obvious is by factor type:

- Hemophilia A patients use factor VIII (FVIII)
- Hemophilia B patients use factor IX (FIX)
- Inhibitor patients use bypassing agents (factor VIIa [FVIIa] or FEIBA)
- VWD patients use factor VIII with VWF (VWF/FVIII complex)

All factor concentrates are also classified by the source of the factor:

- Plasma or
- Cell cultures (*recombinant* factor)

Plasma-derived factor (sometimes abbreviated *pd*) is extracted from human blood plasma collected and pooled from tens of thousands of donors.

Recombinant factor does not come from blood. It's produced by using recombinant DNA technology, in which the gene for a human clotting factor is spliced into another cell

(usually a hamster cell). Large numbers of these genetically engineered cells are then grown in giant vats (bioreactors) containing a nutrient-enriched broth (growth medium). The cells secrete the factor into the growth medium, which is then harvested, and the factor is extracted. An *r* placed before the factor type indicates that it's recombinant: rFVIII, rFIX.

Recombinant FVIII is also classified as either *full length* or *B-domain deleted* (BDD). Full-length factor VIII consists of the entire factor VIII molecule. BDD factor VIII has the center section of the molecule (the B domain) removed; the B domain is not necessary for activity of the factor molecule. BDD factor is shorter than full-length factor and is easier for the genetically engineered cells being grown in the bioreactors to produce.

Finally, recombinant factors are classified into three *generations*, based on whether they contain extra human or animal proteins. First-generation recombinant factors contain added human and/or animal proteins in both the growth medium and the final product.<sup>1</sup> Second-generation recombinant factor concentrates contain no added human or animal proteins in the final product, but do use these proteins in the growth medium. Third-generation recombinant factor concentrates contain no added human or animal proteins in either the growth medium or the final product.

1. In most first-generation recombinant factor concentrates, such as Recombinate, human albumin from blood plasma is typically added to the final container to increase bulk and stabilize the factor.