European Federation of Pharmaceutical Industries and Associations

November 20, 2012

Dr. Kouki Takahashi, Leader, Blood Services Section, Pharmaceutical Affairs and Food Sanitation Council
Dr. Makoto Handa, Chairperson, Blood Services Section Steering Committee
Dr. Masami Kato, Manager, Blood and Blood Products Division, Pharmaceutical and Food Safety Bureau, Ministry of Health, Labour and Welfare

Position Paper concerning "Basic Policy for Improvement of the Safety of Blood Products and Securing Stable Supplies"

Introduction

We have shown the challenges faced by the Japanese plasma derivative business and their countermeasures in the proposal [1)] concerning the "State of Plasma derivative business in Japan," submitted on September 9, 2011 to the Blood Services Section of the Pharmaceutical Affairs and Food Sanitation Council and the Blood and Blood Products Division, Pharmaceutical and Food Safety Bureau, MHLW. We have also submitted an opinion paper concerning the "Review Committee of the State of Supply of Plasma Derivatives: Final Report" [2)] on July 23, 2012, presenting specific opinions and countermeasures based on the content of the committee's final report. At the same time, we noted the discrepancy between the documentation quoted as an indication of citizens' and blood donors wishes concerning blood services [3) 4)], and the outcome of our own studies of citizens and donors, and, presented our report of the study outcomes [5)].

We have pointed out shortcomings in the Japanese blood services policy's information sharing with citizens, blood donors, patients, healthcare professionals and service providers, and as part of our efforts to improve the said situation, hosted a seminar on blood plasma manufacture for the media on July 23, 2012, where patients, clinicians and service providers met under one roof to exchange views with the media. As a result, this event was reported in

c/o GlaxoSmithKline K.K.
GSK Bldg. 6-15, Sendagaya 4-chome, Shibuya-ku, Tokyo 151-8566 Japan
Tel: +81-3-6301-3066 Fax: +81-3-3479-4782

the media in a simple article stating the citizens' point of view of the current challenges for plasma derivative services.

The social and healthcare environments that surround plasma derivatives are now changing considerably, while R&D in plasma derivatives and gene recombination products manufactured with the latest technology have advanced both in Japan and overseas. We believe that we have to urgently review the current issues of plasma derivative business policies, from the viewpoints of citizens, patients and donors.

Following the revision of the basic policy in the title, an MHLW-proposed revision was presented to the Blood Services Section Steering Committee. This proposed basic policy directs the government policies on blood services for the next 5 years. However, it was unfortunately difficult to see any evidence of the issues and solutions we had included in that proposal. In addition, we could not grasp the details of the discussions that precluded the formulation of the proposal. Therefore we have decided to submit this document to request more specific discussions with the blood service providers.

Gist of our requests

The plasma derivatives and blood substitutes gene recombination products we supply make up 41% of Japan's plasma derivative market, based on drug price. These include unique preparations in certain classes or with specific effects used to treat orphan diseases such as Factor VII deficiency, thrombasthenia, Factor XIII deficiency, hereditary angioedema and inhibitor-induced hemophilia, etc. We also supply gene recombinant Factor VIII used in hemophilia A treatment in Japan. We are proud of our sizable contributions to the Japanese plasma derivative business, including the treatment of orphan diseases.

Furthermore, reviews of yet to be approved or off-label use drugs with high clinical needs strongly requested by clinicians' societies are ongoing at the government sponsored "Review Council On Yet To Be Approved or Off-Label Drugs With High Clinical Needs," including blood products: three yet-to-be-approved drugs and two off-label use drugs that concern us. We endeavor to eliminate the drug lag so that Japanese patients can have access to the latest treatment. In addition, we are preparing for the early introduction of next generation gene recombinant coagulation factor substitutes, expected to improve the QOL of hemophiliac patients, to the Japanese market.

We consider it our duty to take part in the discussions on building a framework of sustainable and stable future supply of safe and efficacious drugs from the patients' point of view.
That is why we make the following two requests on the proposed basic policy.

1. To clearly state, "Specific review of the exclusion of plasma derivatives from those listed as subject of the provisions of the Export Trade Control Order shall commence."

2. To clearly state, "As a specific measure to secure domestic self-sufficiency and a stable supply, as stated in The Blood Act (Act on Securing a Stable Supply of Safe Blood Products), fundamental review, such as the introduction of a new blood collection organization specialized in harvesting source plasma for plasma derivatives, in addition to the current blood collection organization, which is a single company, shall commence."

Details of our requests

1. To clearly state, “Specific review shall commence concerning the exclusion of plasma derivatives from those listed as items subject to the provisions of the Export Trade Control Order”

   <Main reasons>

   i. There has been the situation that part of the active blood components obtained through blood donation becomes excessive and is not effectively used, because of the increased supply of gene recombination products and some products that cannot be produced with the manufacturing technologies available to the domestic service provider, etc. By exporting the excess blood components and products that are not needed in Japan to overseas, will be significant to patients who have no current access to the products or sufficient treatments, also in terms of the international contribution our donors make, as well as from the viewpoint of promoting effective use of donated blood and ensuring the level of manufacturing capacity of the domestic service provider. Therefore, plasma derivatives have to be excluded from the list of subjects of the said Export Trade Control Order.

   ii. The backdrop of listing of plasma derivatives in the 1966 Export Trade Control Order was the then controversy surrounding the alleged military use of citizens’ blood for the Vietnam War and other conflicts, which raised an ethical issue, leading to their inclusion in the said list of Control Order items. The current social environment is considerably different from those times, and current Japanese blood services are already implemented under the State’s management. It is hard to imagine that such an issue would arise again.

2. To be specific, “As a specific measure to secure domestic self-sufficiency and a stable supply, as stated in The Blood Act, a fundamental review, such as the introduction of a new blood collection organization specialized in harvesting source plasma for plasma derivatives, in addition to the current blood collection organization, which is a single company, shall commence.”

   <Main reasons>

   i. It has been pointed out that the low birth rate trend and population aging will lead to future shortage of, not only the blood components for transfusion, but also the source plasma for plasma derivatives.[6)] We need fundamental discussions.

ii. The domestic “self-sufficiency” stated in The Blood Act does not require that domestic demands should be supplied by products manufactured by domestic manufacturers, but that the demands will be met with products manufactured from blood donated within the country. At present, there is a demand for 1,500,000 L. We believe that it is necessary to secure further stock of some hundreds of thousands of liters of extra source plasma all the time, as a risk mitigation measure to secure a stable supply during a crisis.

iii. One blood collection organization collects the blood as a source of blood products for transfusion and of plasma derivatives. This approach poses an issue for a stable supply at normal times or in a crisis. We need to avoid the situation whereby a shortage of the source of blood components for transfusion coincides with a shortage of source plasma for plasma derivatives by taking risk mitigation measures.

iv. One approach to dramatically improve the domestic self-sufficiency is to export an equivalent amount of the source plasma collected by domestic blood donation as the currently imported products for processing at overseas plasma derivative business plants. The manufactured derivatives are then re-imported for domestic use. In short, by replacing the source plasma for imported derivative products with domestic sources, the purpose of domestic self-sufficiency is almost attained. We believe this is the most realistic approach. It also secures multiple routes of supply, another measure to mitigate the risk of a stable supply. We believe, however, that the Export Trade Control Order currently prevents this approach from being taken, making it difficult to achieve domestic self-sufficiency.

Thank you
Philippe Fauchet
Chairman
EFPIA Japan

Reference

(1) Proposal concerning “State of Plasma derivative business in Japan”

(2) Opinion concerning the “Review Committee on the State of Supply of Plasma Derivatives: Final Report”

(3) Michio Matsuzaki “Study on the establishment of a system to monitor blood transfusion adverse reactions at medical institutions,” an assigned study as part of “Research into collaborations by medical institutions at discovery of an infection from a product derived from one of the specified organisms,” MHLW Science Research Project for 2005

(4) “Blood donation and blood products study on the recognition and awareness of living citizens” (QLife, Inc., August 25, 2010)

(5) Toshiki Mano, “Challenges for Japanese blood services – discussions based on the results of a questionnaire survey of citizens and blood donors” Japan Medical Journal No. 4570, November 26, 2011

(6) “Future population of Japan and chronological changes in the potential blood donating population” Resource for Blood donation promotion investigation team, Blood Services Section, Pharmaceutical Affairs Working Group, Pharmaceutical Affairs and Food Sanitation Council, first meeting in 2010

Contact

Noriaki Kanemoto
Secretary General
European Federation of Pharmaceutical Industries and Associations (EFPIA) Japan
ZIP code 151-8566
GSK Building, 4-6-15, Sendagaya, Shibuya-ku, Tokyo

Phone: 03-6301-3066 (representative & secretary general)
Fax: 03-3479-4782

Makoto Miyakawa
Leader
Blood Product Taskforce
(Operation and Planning, President's Office, CSL Behring, Co. Ltd.)
Phone: 03-3534-5906
Fax: 03-3534-5864
Email: Makoto.Miyagawa@cslbehring.com