Editorial

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Collaboration through the Asia Pacific MPS Network (APMN), Asia Pacific MPS Registry (APMR), and Association for Research of MPS & Rare Diseases (ARMRD)

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Though the rate of incidence of each rare disease, including mucopolysaccharidosis (MPS), is low, this is not the case if they are taken as a whole. Rare diseases often have genetic causes and vary in type. However, the signs and symptoms vary greatly by disease, making it difficult to make accurate diagnoses and conduct necessary research, which is why we believe it is a field that deserves more attention and research. It is important to establish an infrastructure of experts in each country and promote cooperation within the Asia-Pacific region in order to improve specialist training and communication. Given the need for a system of cooperation, the Asia Pacific MPS Network (APMN) was established by several MPS experts in South Korea, Japan, and Taiwan in January 2013. Thereafter, the Asia Pacific MPS Registry (APMR), an electronic remote data system, was established by the APMN. Then, the Association for Research of MPS & Rare Diseases (ARMRD), an academic society that supports research on MPS and other rare diseases, was established by President Dong-Kyu Jin in April in 2015. The main task of the ARMRD is to support APMN-related work. The ARMRD published a uniform guideline that reflects the characteristics and circumstances of local patients through the Korean MPS Expert Council. Now, the APMN, APMR, and the annual Korean MPS Symposium are supported by ARMRD. Organizations like the APMN and APMR are necessary because international cooperation and collaboration are needed to conduct clinical trials on those diseases. ARMRD members hope to encourage the interest of experts and researchers of MPS & rare diseases as well as active participation in the research and treatment of patients suffering from rare diseases, including MPS, to ultimately improve the quality of life of the patients as well as their families.

Keywords: Mucopolysaccharidosis, Rare diseases, APMN, APMR, ARMRD

Introduction

Though the rate of incidence of each rare disease, including mucopolysaccharidosis (MPS), is low, this is not the case if they are taken as a whole. Rare diseases often have genetic causes and vary in type. However, the signs and symptoms vary greatly by disease, making it difficult to make accurate diagnoses and conduct necessary research, which is why we believe it is a field that deserves more attention and research. Due to the persistent effort to overcome such obstacles, there have recently been great developments in Korea in terms of the research, diagnosis, and treatment of rare diseases, including MPS. In particular, the study of MPS has developed to meet global standards.

Academic collaboration has begun among specialists in MPS care in the Asia-Pacific region, which includes Japan, Taiwan, Malaysia, and South Korea, via an organization called the Asia Pacific MPS Network (APMN). Via the APMN, doctors and researchers who are interested in the treatment of patients with MPS collaborate and exchange information, and they present basic and clinical research related to MPS. The APMN developed an MPS-specific registry a year after its establishment. Now, the APMN, APMR, and annual Korean MPS Symposium are sup-

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ported by the academic society called the Association for Research of MPS & Rare Diseases (ARMRD). Here, I introduce the collaboration between the APMN and APMR, and ARMRD.

1. The Asia-Pacific MPS Network (APMN)

Although individually rare, MPS disorders collectively are highly prevalent, with an overall incidence of 1:22,000–52,000. Asia is the world's most populous continent, with approximately 4.3 billion people. Many patients may have gone undiagnosed or may be receiving minimal care in this region. In fact, in most countries, there is a lack of specialized healthcare personnel to provide adequate, comprehensive care for patients with MPS. Although the management of some clinical problems associated with MPS may seem routine, management is usually complicated and requires physicians' awareness of issues specific to the disease. Therefore, a multidisciplinary approach and the coordination of efforts by the professional, social, and governmental sectors are required.

It is important to establish an infrastructure of experts in each country and promote cooperation within the Asia-Pacific region in order to improve specialist training and communication. Based on this need, the APMN was established by several MPS experts in South Korea, Japan, and Taiwan in January 2013. The four main objectives of the APMN are as follows: 1) to organize an Asia-Pacific research network for MPS (establishing a registry); 2) to understand the current state of the disease and exchange information on it; 3) to provide support for preclinical studies related to MPS and patients with MPS/parents in the Asia-Pacific region; and 4) to encourage and engage in an international exchange of younger doctors who are treating MPS patients. A survey to assess the current patterns of MPS diagnosis and treatment in the Asia-Pacific region should be conducted to identify gaps in knowledge and unmet needs that must be addressed and to provide opportunities for research on particularly relevant topics in the region. The professional knowledge and experience of MPS experts should be shared through international cooperation in order to provide better treatment to patients with MPS in Asia, in the Pacific region, and around the world. All of these efforts should be aimed at providing effective care for MPS patients. Recently, the APMN has been expanding through the participation of other Asian experts from Malaysia and Vietnam.

2. The Asia-Pacific MPS Registry (APMR)

One of the obstacles to the identification, understanding, and treatment of MPS is the relative paucity of information. Therefore, regional and national disease registries are needed to facilitate better understanding of the natural history of this clinically heterogeneous disease and to accrue long-term data to evaluate existing and new therapies. One way in which patients can take part in ongoing research efforts is through their enrolment in disease-specific registries. Observations based on these international registries will provide insights into the natural history of the disease and the long-term effects of various therapies. Such data can be used to help identify unmet patient needs and encourage further research. Registries have been established for MPS type I (www.mpsiregistry. com), MPS type II (www.elaprase.com/patients_ families/about_hunter/outcomes/), MPS type IVA, and MPS type VI (http://www.naglazyme.com/en/ Clinical-resources/surveillance-program.aspx); the Morquio A Clinical Assessment Program has also been initiated. However, these registries are managed by pharmaceutical companies that make drugs for MPS patients. Moreover, they mainly include patients from Europe and the US, and most Asian patients with MPS have not been enrolled in these registries. The registration of patients and subsequent epidemiological research should be independent of any pharmaceutical and side effects of ERT drugs. In this regard, the APMR, an electronic remote data system, was established by key doctors in the APMN.

The healthcare systems in individual Asian-Pacific countries differ greatly in terms of their structure and access to diagnostic tools and treatment. Furthermore, there are large variations in insurance coverage in different countries, and even in regions within the same country. In countries where ERT is not currently available, symptomatic management remains the primary treatment option. There is also substantial national variation in patterns of MPS monitoring. The APMR has been structured to accommodate different clinical practices in different countries/regions because data related to procedures that are not routinely performed in a particular country are sometimes requested. The APMR also includes patients who have not received ERT. The APMR will include patients with all types of MPS and it will act as a hub in global clinical trials of any new drugs for patients with MPS. The registration of patients with Hunter syndrome began in July 2014, and the APMR will be expanded into a global MPS registry (GMR) as the number of nations participating in the APMN increases. The APMR can be used for other rare diseases in the future.

3. The Annual Korean MPS Symposium

The first Korean MPS Symposium was held in May 2002, while the most recent, the 14th Korean MPS Symposium, was held in May 2015. Initially, the symposium was more of a domestic conference. However, the scale of the symposium has expanded, and speakers and attendees now come from around the world. The symposium consists of two sessions – a scientific session and a session for patients and families. In the academic session, many global experts on MPS present clinical studies and up-to-date results from laboratory research. At the symposium, attendees are able to engage in detailed discussions, and they have the opportunity to share their experiences with global experts. The family session provides time for family support in various areas and for Korean families of patients with MPS to become acquainted. This assembly offers support and advocacy for patients and family groups. The 15th Korean MPS symposium will be held alongside the 8th APMN meeting at Jeju Island in May 2016. This joint meeting will be supported by ARMRD.

4. Association for Research of MPS & Rare Diseases (ARMRD)

ARMRD is an academic society that supports research on MPS and other rare diseases, and is established as a corporate body. The ARMRD was founded to support collaborative research on MPSs and rare diseases. It was established by President Dong-Kyu Jin in April in 2015. The headquarters of the ARMRD is located in Seoul, Korea. The main task of the ARMRD is to support APMN-related work; objectives of the ARMRD are as follow.

1) Support for the APMN meeting and annual Korean MPS Symposium
2) Management & activation of APMR (All types of MPSs including Morquio a disease)
3) Management of the ARMRD homepage
4) Support for the MPS- and rare diseases-related symposium
5) Publication of the MPS- and rare diseases-related guideline

Clinical and genetic characteristics and medical conditions related to MPS differ in each country. A standard guideline for Korean patients with MPS was required for the appropriate evaluation of treatments and to guarantee the safety of patients. In addition, evidence of the need for treatment needed to be compiled. In light of these requirements, ARMRD published a uniform guideline that reflects the characteristics and circumstances of local patients through the Korean MPS Expert Council (KMEC). The Korean guidelines for Hunter syndrome and Hurler syndrome were published in May 2014, and in Dec 2015, respectively. Individual guidelines for other subtypes of MPS will be published regularly. Internationally, we have been actively exchanging with rare disease experts in East Asian countries, including Japan, Taiwan, and Malaysia, as the center of the APMN. Through such activities, we are assuming a pivotal role in Asia in terms of the diagnosis and research of rare diseases, including MPS.

Conclusion

Organizations like APMN and APMR are necessary because international cooperation and collaboration are needed to elucidate the mechanisms of rare diseases and to conduct clinical trials on those diseases. The collaboration between Japan, South Korea, and the other Asian countries will be of the utmost importance in the future. ARMRD members hope to encourage the interest of experts and researchers of MPS and other rare diseases as well as their active participation in the research and treatment of such diseases to ultimately improve the quality of life of the patients as well as their families.

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