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Press release

Nippon Shinyaku Co., Ltd.

Biogen Japan Ltd.

Sanofi K.K.

**The "Moshikashite NMD" project is launched.  
Special dance and check tools are developed collaboratively to  
help discover neuromuscular disorders earlier.  
-- Tools for children with rare diseases in whom diagnosis may  
often be delayed --**

Nippon Shinyaku Co., Ltd. (Head office: Kyoto-shi, Kyoto; President: Shigenobu Maekawa; hereinafter "Nippon Shinyaku"), Biogen Japan Ltd. (Head office: Chuo-ku, Tokyo; President: Ajai Sulekh; hereinafter "Biogen"), and Sanofi K.K. (Head office: Shinjuku-ku, Tokyo; President: Takahiko Iwaya; hereinafter "Sanofi") today announced that the three companies have developed check tools that help parents, childcare workers, and teachers notice early signs of rare diseases (e.g., Duchenne muscular dystrophy [DMD], spinal muscular atrophy [SMA], and Pompe disease) in children in whom an early diagnosis is often difficult. This set of check tools includes a dance that is enjoyable for all young children and is helpful for their caregivers to notice early signs based on their slight differences in movement. This project, named "Moshikashite NMD" (Could this be NMD?), will be promoted through a dedicated website (<https://moshikashite-nmd.jp/>) and paper media.

\* NMD = neuromuscular disorders



Prior to the development of these check tools, people living with DMD, type III SMA, and Pompe disease were interviewed online about their typical signs and symptoms. Based on these interviews, the three companies developed check tools under supervision by Dr. Hirofumi Komaki, the director of the Translational Medical Center at the National Center of Neurology and Psychiatry.

The check tools consist of "the NMW Quiz" and "the NMD Check Dance"

- NMD Quiz



●NMD Check Dance



The NMD quiz uses easy-to-grasp illustrations describing typical early signs and symptoms of NMDs and short questions asking whether these signs and symptoms are noted or not. Mr. Neji Sato, the president of **Blue Puddle Inc.**, a company developing **various** digital contents, has contributed to the creation of the quiz.

The NMD **check** dance, named "Ai-Yeah Jumbo!", was developed as a dance that is enjoyable for all children and is helpful for caregivers to notice early signs of NMDs. Mr. Yoshihisa Kobayashi, the previous "Taiso no Oniisan" (dance navigator) for NHK's popular child program "Okaa-san to Issho" (Play with Mom), **danced** for the video and **supervised the program**. Mr. Kunihiro Taniguchi (**Tanizo**), who has created many songs and dances including "Boom Ba Boink!" for the Play with Mom program, wrote the song and choreographed the dance.

The three companies will publish the NMD check tools on their websites and publish paper-based materials to help parents as well as caregivers and teachers at day-care centers, kindergartens, and elementary schools.

Dr. Hirofumi Komaki, the supervisor of the NMD check tools, said, "Almost all of the differences in children's physical capabilities are explained by their personality. However,

when some of these differences persist or gradually get large, these may be caused by a neuromuscular disorder (NMD) in rare cases. As NMDs are progressing diseases, it is critically important to visit clinics and diagnose the disease as early as possible. Some children with NMDs have been suspected to have bone diseases and have not been diagnosed as having neurological disorders for a long period of time. It is also important to visit specialist clinics. Medical technology has advanced, and an increasing number of diseases have become treatable. We developed these easy-to-understand tools to help parents, caregivers, and teachers, as well as people surrounding the children to notice early signs of NMDs. We hope our NMD check tools will raise awareness of the importance of the early discovery of rare diseases."

For details about the "Moshikashite NMD" project, please visit the following website:

<https://moshikashite-nmd.jp/dance/>

### **About Duchenne muscular dystrophy (DMD)**

Duchenne muscular dystrophy (DMD) is an inherited muscle disorder that male children mainly develop. It causes a loss of muscle power due to a deficiency of dystrophin, a protein involved in constructing the framework of muscle cells. Patients show delays in walking and other motor development. Their physical capabilities peak at around the age of five and then show a gradual decrease in their motor functions. There is a progressive loss of mobility, and by adolescence, patients with DMD may require the use of a wheelchair. Cardiac and respiratory muscle problems begin in the teenage years and lead to serious, life-threatening complications.

### **About spinal muscular atrophy (SMA)**

Spinal muscular atrophy (SMA) is a disorder characterized by the progressive loss of motor neurons in the spinal cord and the lower brain stem, which causes serious and progressive muscular atrophy and weakness. When untreated, patients with the most serious type of SMA may become paralyzed and lose physical functions such as respiration and swallowing to maintain life.

SMA develops due to the loss or mutation of the *SMN1* (survival of motor neuron 1) gene. Patients with SMA cannot produce a sufficient amount of SMN protein, which is necessary for the maintenance and survival of motor neurons. The severity of SMA correlates with the amount of SMN protein an individual has. People with infantile SMA, the form that requires the most intensive and supportive care, produce very little SMN protein and, if not treated,

do not achieve the ability to sit without support or typically live beyond two years without respiratory support. People with other types of SMA produce greater amounts of SMN protein and have less severe, but still life-altering, forms of SMA.

### **About Pompe disease**

Pompe disease is an inherited metabolic disorder caused by insufficient breaking-up of waste materials in cells. Pompe disease is caused by the lack or deficiency of enzymes that break up glycogen (complex sugars) in muscles and other tissues and organs, and patients with this inherited condition experience a variety of symptoms, including loss of muscle strength. Pompe disease is classified into infantile-onset Pompe disease, which presents in the first few months after birth, and late-onset Pompe disease, which develops in childhood or adulthood. Infantile-onset Pompe disease is characterized by cardiac hypertrophy and generalized muscle weakness and may lead to death in several months to years after birth. Late-onset Pompe disease is characterized by muscular weakness and may develop at any age from children to the elderly, causing various complications.

Pompe disease has been designated as an intractable disease in Japan. As the treatment for Pompe disease has been established, it is important to diagnose and start treatment at an early stage to prevent its progression and the development of complications.

### **About Nippon Shinyaku**

Nippon Shinyaku's mission is to help people lead healthier and happier lives. Intractable and rare diseases are one of our four focusing therapeutic areas. In recent years, we have been dedicated to the development of nucleic acid medicine and obtained a marketing approval of the DMD's agent for the treatment of exon 53 skipping for the first time in Japan this year. We are now conducting its information activities to medical institutions. We continue to fulfil development pipeline of DMD and aim to be an organization trusted by the community through creating unique medicines that will bring hope to patients and families struggling with DMD. Please visit our website (<https://www.nippon-shinyaku.co.jp>) for our detailed information.

### **About Biogen**

At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies. One of the world's first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann,

Heinz Schaller, Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today Biogen has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, commercializes biosimilars of advanced biologics, and is focused on advancing research programs in multiple sclerosis and neuroimmunology, Alzheimer's disease and dementia, neuromuscular disorders, movement disorders, ophthalmology, immunology, neurocognitive disorders, acute neurology, and pain.

We routinely post information that may be important to investors on our website at <https://www.biogen.co.jp/>. Follow us on social media – [Twitter](#), [Facebook](#), [Instagram](#), [YouTube](#).

### **About Sanofi**

Sanofi is dedicated to supporting people through their health challenges. We are a global biopharmaceutical company focused on human health. We prevent illness with vaccines, provide innovative treatments to fight pain and ease suffering. We stand by the few who suffer from rare diseases and the millions with long-term chronic conditions.

With more than 100,000 people in 100 countries, Sanofi is transforming scientific innovation into healthcare solutions around the globe.

Please visit the company's website (<http://www.sanofi.co.jp>) for details of Sanofi K.K.