

International survey for Rare Diseases PG/PAGs

*実際にはWebフォームを使って実施

*必須

About BASIC INFORMATION;

1-1. Please describe your organization name *

1-2. When your organization was establishment? *

2. What kind (s) of your target disease(s)? *

*Please describe individual name of target disease(s). If your target diseases are all RD, please also describe "all Rare Disease" etc.

1-3. What kind (s) of your constituent member? *

*Please check all that apply

Patient

Patient family

Supporter (professional)

Supporter (general)

everyone

only PG/PAGs

others

1-4. How many member (individual) do you have? *

*If you have information/articles/data related to this question on your website, you don't need describe and please put the web address in the following column.

1-5. In your country, how many organizations (PG/PAGs) as member do you have? *

*If you have information/articles/data related to this question on your website, you don't need describe and please put the web address in the following column.

1-6. Out of your country, how many organizations (PG/PAGs) as member do you have? *

*If you have information/articles/data related to this question on your website, you don't need describe and please put the web address in the following column.

1-7. Is the number of membership bigger than 5 years ago? *

Yes, bigger than 5 years ago

No, smaller than 5 years ago

Stable

1-8. What do you think why the number of membership has changed/not changed in your

organization?

1-9. Please let us know about your constituent management organization. *

*Please check all that apply

- Annual meeting
- Board meeting
- Management committee
- Council
- others

1-10. Please let us know about your CURRENT activities. *

*Please check all that apply

- Board training
- Caregiver/Nurse program
- Community workshop
- Conference
- Corporate with companies
- Collaborate with researchers/physicians
- Database
- Education to High school
- Education to University
- Fundraising
- General assembly
- Help lawsuit
- Information center (to patient)
- Information center (to professional)
- Investment
- Lecture
- Lobbying
- Media advertisement
- Online patient community
- Patient meeting
- Petition
- Publication
- Public awareness campaign/event
- R&D support
- Request donation
- Request supporting money

Research fund
School management
Signature collecting campaign
SNS/blog
Sponsored research by national government
Sponsored research by local government
Sponsored research by industries
Website
Volunteer training
Others

1-11. Please let us know about your MAIN activities. *

*Please select and describe 3-5 activities from above items

1-12. Please let me know about your activities at the time of establishment. *

*Please check all that apply

Board training
Caregiver/Nurse program
Community workshop
Conference
Corporate with companies
Collaborate with researchers/physicians
Database
Education to High school
Education to University
Fundraising
General assembly
Help lawsuit
Information center (to patient)
Information center (to professional)
Investment
Lecture
Lobbying
Media advertisement
Online patient community
Patient meeting
Petition
Publication

Public awareness campaign/event
R&D support
Request donation
Request supporting money
Research fund
School management
Signature collecting campaign
SNS/blog
Sponsored research by national government
Sponsored research by local government
Sponsored research by industries
Website
Volunteer training
Others

1-13. How many full-time staff do you have? *

1-14. How many part-time staff do you have? *

1-15. What kinds of staff do you have? *

*Please check all that apply

Patient
Patient related persons
Professional (medical)
Professional (business)
Professional (marketing)
Professional (IT)
Student intern
Others

1-16. Please let us know about your income and expenditure. *

*Please describe in detail. If you have information/articles/data related to this question on your website, you don't need describe and please put the web address in the following column. If you have a data and be able to disclosure, please e-mail to Yukiko;

About RESEACH COLLABORATION;

2-1. Does your organization support to research? *

Yes
No
Ohters

2-2. What type of Research collaboration have you continued to?

*Please describe in detail. If you have information/articles/data related to this question on your website, you don't need describe and please put the web address in the following column.

2-3. Do you think what kinds of services/supports are suitable for PGs those are able to easy to collaborate with researchers/physicians?

*Please check all that apply

Provide traffic/hotel expenses

Provide communication cost

Explanation about the research

Discussion with researcher(s) and/or industry

Set the meeting with/among related persons

Others

About KNOW-HOW;

3-1 How to get staff? (Volunteer / employee) *

3-2 How to share rich information with member? (before / after internet diffusion) *

*Please describe before / after internet diffusion.

3-3 How to engage in educational activities for elderly patients who cannot use the internet very well?

3-4 How to collaborate with other local/international PGs?

3-6 When you manage the patient organization, what' the point(s) which you attach particular importance on? *

About PATIENT REGISTRY;

4-1. Do you have the patient registry project? *

Yes

No

4-2. If you have already released your patient registry, please put the website address.

4-3. What the type of people can register?

*Please check all that apply

Patient

Physician

Patient under physician's advice

Others

4-4.What's the purpose of building Patient Registry?

*Please check all that apply

Conduct a thorough investigation to determine the cause

Cohort research

Developing new drug/product

Evaluate medical economy

Evaluate medical quality

Investigate natural histories

Make/evaluate diagnostic standard

Marketing support

PMS (Post Marketing Surveillance)

PMS (Post Marketing Surveillance) Understand disease rate etc

4-5. How many CURRENT registers do you have?

4-6. How to get correct data with keep respondents' motivation?

4-7. Do you have any relations with foreign registries?

Yes

No

4-8. What kinds of collaboration do you have with foreign registries?

4-9. Do you create any ingenuities for building registry? If you have, please describe follow.

4-10. Do you have any troubles for building registry? If you have, please describe follow.

If it is possible, please describe your name and e-mail address.

*If we have any questions related to this survey, we would like to contact to you. We also will report this survey to you.

MEMORANDUM OF UNDERSTANDING

Japan Patients Association (JPA)
and the
National Organization for Rare Disorders (NORD)

January 8, 2013



**JAPAN PATIENTS ASSOCIATION (JPA) AND
THE NATIONAL ORGANIZATION FOR RARE
DISORDERS (NORD)
MEMORANDUM OF UNDERSTANDING**

WHEREAS, the Japan Patients Association (JPA) and the National Organization for Rare Disorders (NORD) have come together to collaborate to improve the lives of patients affected by rare and intractable diseases and living in Japan or the USA; and

WHEREAS, the partners listed below have agreed to enter into a collaborative agreement to work together in areas identified as of mutual interest; and

WHEREAS, it is becoming increasingly evident that addressing challenges related to rare and intractable diseases often requires international collaboration; and

WHEREAS, patients, their families and patient organizations affected by rare and intractable diseases often share similar challenges even though they may live in different parts of the world;

THEREFORE, the Japan Patients Association (JPA) and the National Organization for Rare Disorders (NORD) do issue this declaration of shared commitment to improving the lives of the patients and families they represent and declare their intention of identifying areas in which to collaborate on initiatives related to advocacy, education, awareness and other topics of importance to the rare and intractable disease community.

**日本難病・疾病団体協議会 (JPA) ならびに米国
希少疾病協議会 (NORD) 間の連携に関する覚書**

JPA と NORD は、日本およびアメリカ合衆国に在住する希少・難治性疾患患者の生活を改善することを目的としてともに協力します。

以下に述べる両組織は、双方が関心を持つ特定領域において連携するため、連携契約を締結することに合意します。そして、希少・難治性疾患領域で頻繁に求められる国際連携に関する挑戦課題を明らかにしていきます。

この場合、課題とは、たとえ世界の異なる地域に住んでいる場合であっても、希少・難治性疾患領域の患者、患者家族、ならびに患者会がしばしば行っている同様の挑戦を指します。

従って、日本難病・疾病団体協議会 (JPA) ならびに米国希少疾病協議会 (NORD) は、患者および患者家族の生活改善に関する共通課題を記した本宣言に署名します。本宣言では、希少疾患を取り巻く社会において重要な、患者支援、教育、意識向上およびその他の連携するべき取組領域を特定する意志を明らかにしたうえで公表します。

Description of Partner Agencies

The Japan Patients Association (JPA) is a non-profit organization established in 2005. JPA is an umbrella association related to "NANDYO", rare and intractable diseases. Total membership of JPA is about 300,000 from 72 organizations including individual patient groups and regional centers (as of 2012). The mission of JPA is "Construct a people-oriented medical treatment and welfare social system" and "Try to establish Japan's National Center". JPA provides patient/family services, lobbying to the government, petition to the congress, and so on. JPA also collaborates among other associations in Japan and has organized patients' forums since 2012.

The National Organization for Rare Disorders (NORD) is a non-profit, 501(c)3 organization established in 1983. It serves as the primary advocacy organization for the 30 million Americans with rare diseases and those seeking to improve their lives. NORD provides programs of advocacy, education, research and patient/family services through its offices in Washington DC; Danbury, CT; and Quincy, MA. It also provides mentoring for rare disease patient organizations and particularly for its 200 member organizations, and seeks to enhance physician and public awareness of rare diseases and of the challenges of living with a rare disease.

Roles and Responsibilities

Initially, the collaboration between JPA and NORD will focus on general activities designed to increase understanding and communication between rare disease patients and patient organizations in Japan and the USA. Such activities will include:

- Sharing information about rare disease patient

organizations in Japan and the USA

- Sharing information about current advocacy initiatives in Japan and the USA
- Displaying the logo of the partner organization, with a statement of collaboration and friendship, on each organization's website
- Issuing a joint press release about the collaboration between the two organizations

Commitment to Partnership

We, the undersigned, have read and agree with this MOU. We approve this declaration of friendship and collaboration, and declare our intent to investigate other, more formal ways of working together in the future.



Peter L. Saltonstall ビーター・サルトンストール

President and CEO, NORD
NORD 代表兼 CEO

2013 年 1 月 8 日

January 8, 2013

双方の患者協議会について

JPA は、2005 年に設立された希少・難治性疾患領域患者協議会であり、2011 年からは一般社団法人として活動しています。加盟組織は、個別患者会ならびに地域難病連であり、2012 年 12 月現在で 72 組織、約 30 万人が活動しています。当該領域における豊かな医療と福祉の実現を目指しており、National level の患者協議会として、情報発信、政策提言、政府委員会参加等を積極的に実施しています。近年では、他の患者会を束ねたフォーラム開催等もっており、患者意見の共有・統一を目指しています。

NORD は 1983 年に設立した非営利機関です。NORD は、希少疾患患者および彼らの生活改善に向けた支援をしている国内約 3000 万人に対する主要な支援組織です。NORD は支援、教育、研究および患者/患者家族へのサービスもしくはプログラムを実施しております。オフィスはワシントン DC、ダンバリー（コネチカット）、クインシー（マサチューセッツ）の 3 か所にあります。さらに、NORD は会員である 200 の患者会および患者支援組織に対し、認知度向上や研究者支援を目指す患者会への相談事業を実施しています。

責任および役割

はじめに、JPA と NORD 間の連携は、日本およびアメリカ合衆国における希少疾患患者と患者会間のコミュニケーションならびに相互理解を深めることを目的とした全般的活動に焦点をあてます。すなわち、

- 日本およびアメリカ合衆国における希少疾患患者会に関

する情報の共有

- 日本およびアメリカ合衆国における支援活動の現状に関する情報の共有
- 双方ウェブサイト上に、両組織の連携および友好関係を明記した書類、ならびに組織ロゴの掲載
- 両組織間の連携に関するプレスリリースの共同配信

パートナーシップに関する誓約

私たちは、本 MOU の内容を確認したうえで同意し、以下に署名します。私たちは友好および連携に関する本宣言を採択します。また、私たちの関心領域を明らかにし、近い未来にさらなる公式的手法によってともに活動する決意をここに表明します。



伊藤たてお Tateo Itoh

President, JPA
JPA 代表理事

(日本語版プレスリリース文書)

報道発表 2013年1月29日

日米希少・難治性疾患患者協議会がパートナーシップを確立
—日本難病疾病・団体協議会 (JPA) と米国希少疾病協議会 (NORD) が連携に
関する覚書を締結—

(ワシントン DC 発)

日米の希少・難治性疾患の患者及び患者家族の会の連合組織である米国希少疾病協議会 (NORD) と日本難病疾病・団体協議会 (JPA) が、患者たちの生活改善に関わる活動に関する連携覚書 (MOU) に署名した。

NORD と JPA 間のパートナーシップは、2013年 (平成 25年) 1月 8日に、NORD 代表兼 CEO のピーター・サルトンストールと JPA 代表理事の伊藤たておによって、ワシントン DC の NORD オフィスにて正式に締結された。

伊藤氏は「私たちは同じミッションそしてゴールを共有しています。両組織が持っている知見や経験を、日米両国にて相互連携のために役立てていきたいと思えます。」と述べた。

「国際連携は、希少・難治性疾患患者に必要な治療法の開発、公共政策の改善および生活の質の向上へとつながります。」サルトンストール氏は述べた。

「NORD と JPA の共通点はとてもたくさんあります。私たちは日米の希少・難治性疾患社会のため、ともに活動することを楽しみにしています。」

本 MOU では、以下のように言及している。「希少疾病領域で頻繁に求められる国際連携に関する挑戦課題を明らかにしていきます。ここで述べた課題とは、世界の異なる地域に住んでいる希少疾患領域の患者、患者家族、ならびに患者会が盛んに行っている同様の挑戦を指します。」

患者および患者家族の「生活改善に関する共通課題を記した本宣言」である本 MOU により締結されたパートナーシップにより、今後両機関は患者支援活動の現状について情報を共有するとともに、日本およびアメリカ合衆国における希少疾患患者会の相互理解および連携を促進させる方法を検討していく。

NORD について

今年設立 30 周年を迎えた NORD は、1983年にオーファンドラッグ法制定の際とともに活動した患者および支援者により 1983年に設立した。NORD は希少疾患社会の利益となるような支援、教育、研究および患者/患者家族へのサービスを実施している。NORD は、希少疾病患者および彼らの生活改善に向けた支援を

おこなっている。また、認知度向上や研究者支援を目指す患者会への相談事業を実施している。NORD は約 200 の会員機関を有している。NORD は JPA との新規パートナーシップに加え、EURORDIS（ヨーロッパ希少疾患団体）とも既に戦略的連携を締結しており、インターネットを通じた国際交流 (www.RareConnect.org) やそのほかの活動を通じて欧米の希少疾患患者間をつないでいる。

JPA について

JPA は、2005 年に設立された希少・難治性疾患領域患者協議会であり、2011 年からは一般社団法人化した。加盟組織は、個別患者会ならびに地域難病連であり、2012 年 12 月現在で 72 組織、約 30 万人が活動している。当該領域における豊かな医療と福祉の実現を目指しており、全国レベルの患者協議会として、情報発信、政策提言、政府委員会参加等を積極的に実施している。2010 年からは、他の患者会を束ねたフォーラム開催等も行っており、患者意見の共有・統一を目指している。

(英語版プレスリリース文書)

FOR IMMEDIATE RELEASE

JANUARY 29 2013

Leading Rare Disease Patient Organizations in U.S. and Japan Establish Partnership

Japan Patients Association (JPA) and National Organization for Rare Disorders (NORD) Sign Memorandum of Understanding

Washington DC-----The primary organizations representing patients and families affected by rare diseases in the U.S. and Japan have agreed to collaborate to improve the lives of their constituents.

The partnership, uniting the Japan Patients Association (JPA) and the National Organization for Rare Disorders (NORD), was formally established at the NORD offices in Washington DC by NORD President and CEO Peter L. Saltonstall and JPA President Tateo Ito.

“Global collaboration will drive progress toward new treatments, improved public policies and better lives for people with rare diseases,” Saltonstall said. “NORD and JPA have much in common. We look forward to working together on behalf of the rare disease communities in Japan and the U.S.”

“I could meet NORD staff each other and clearly imagine the future cooperation with us specifically. “ Ito said. “We would like to make use of our valuable knowledge and experiences for the mutual collaboration in both countries.

JPA is a non-profit umbrella organization established in 2005. It focuses on rare and intractable diseases and has a membership of approximately 300,000 from 72 organizations, including individual patient groups and regional centers. JPA provides patient/family services and advocacy on public policies. It also collaborates with other organizations in Japan and has organized patients’ forums since 2012.

Celebrating its 30th anniversary this year, NORD was established in 1983 by patient advocates who worked together to get the Orphan Drug Act enacted. It provides advocacy, education, research and patient/family services on behalf of the rare disease community. NORD also administers patient assistance programs to help patients access life-saving medications. NORD represents approximately 200 member organizations.

The MOU notes that “it is becoming increasingly evident that addressing challenges related to rare and intractable diseases often requires international collaboration” and that “patients, their families and patient organizations affected by rare and intractable diseases often share similar challenges even though they may live in different parts of the world.”

Calling the partnership a “declaration of shared commitment to improving the lives” of rare disease patients and families, the MOU specifies that the two organizations will share information about current advocacy initiatives and seek ways to increase understanding and communication between rare disease patients and patient organizations in Japan and the U.S.

In addition to this new partnership with JPA, NORD also has a strategic alliance with EURORDIS (Rare Diseases Europe) to connect rare disease patients in the U.S. and Europe through global online communities (www.RareConnect.org) and other initiatives.