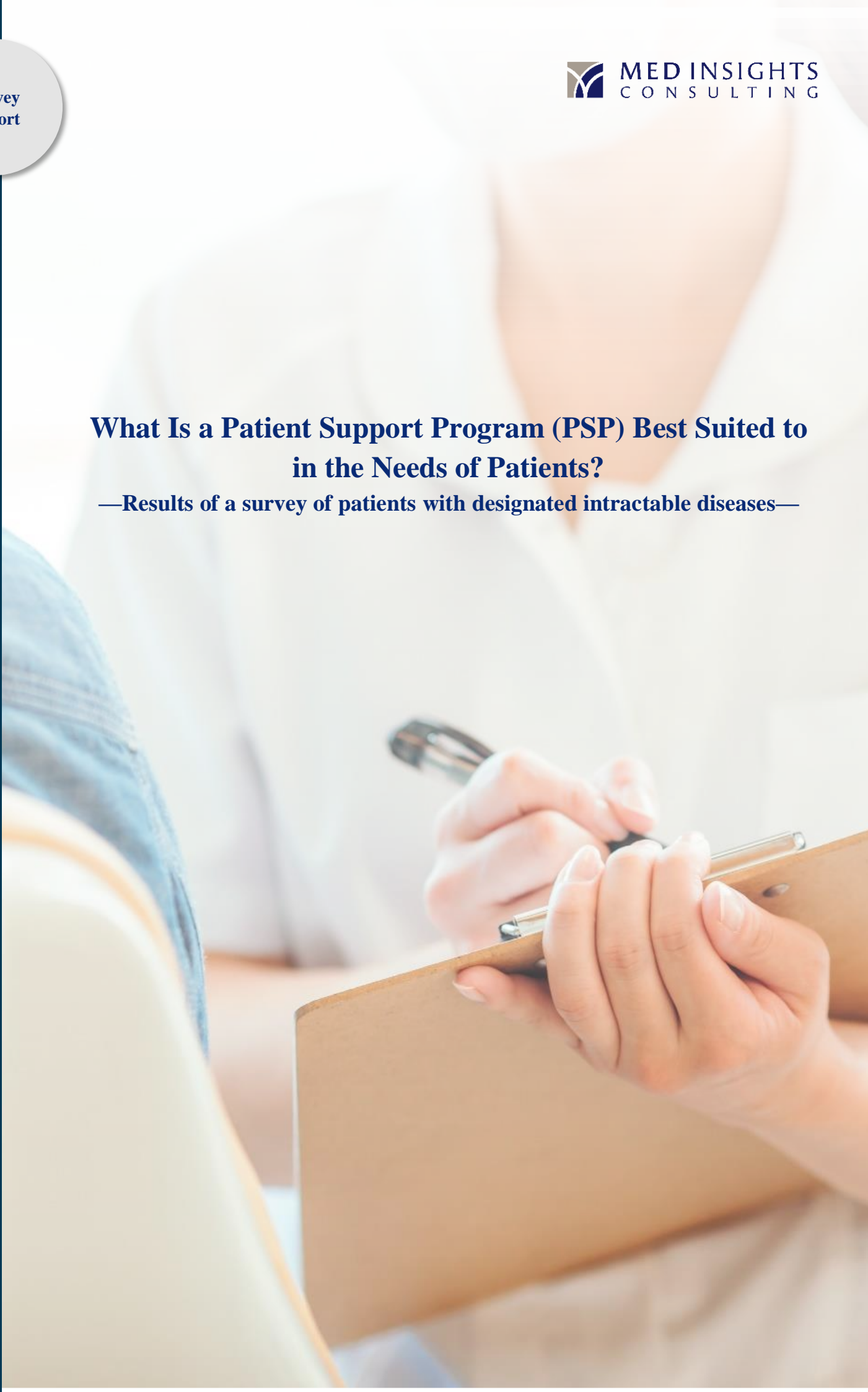


What Is a Patient Support Program (PSP) Best Suited to in the Needs of Patients?

—Results of a survey of patients with designated intractable diseases—



Introduction

Even with new therapies being developed on a daily basis, as well as many medicinal products developed to date, many patients still suffer from intractable diseases and other chronic diseases. In Japan, the number of “designated intractable disease medical care recipient certificate” holders totaled 1,033,700 as of the end of March 2021, while growing steadily.¹⁾ Such designated intractable diseases and chronic diseases lead to a reduced quality of life (QOL) in not only patients, but also their families (caregivers) due to prolonged treatment including drug therapy, mental distress from morbidity, and financial and other burdens. Meanwhile, reduced adherence, such as a failure to follow instructions from the attending physician or discontinuation of medication on the patient’s own judgment, leads to a reduction in drug efficacy, resulting in further prolongation of treatment, doctor shopping, and other problems. These situations are partly attributable to the lack of information about diseases and therapies, and medications used, including their side effects, as well as a lack of understanding of such information.

To address such problems, pharmaceutical companies in Europe and the United States commonly provide patient support programs (PSPs) to increase patients’ adherence and awareness of diseases, and provide support on the usage of specific medicinal products and support against anxiety in patients and their caregivers during treatment. It has been reported that education on disease and information useful for home care that had been provided through these programs led to increases in adherence and QOL, indicating the usefulness of PSPs.

In Japan, similar services such as “Okusuri Soudan” (drug consultation) had been provided, which failed to solve the problems associated with a reduced QOL in patients with designated intractable diseases and their caregivers. Thus, some PSPs for these diseases have commenced.

We conducted a questionnaire survey of patients with designated intractable diseases, aiming to obtain an understanding of patients’ needs which would be required to provide a PSP best suited to patients and their caregivers in Japan. We sincerely hope that these data will contribute to the spread and improved quality of PSPs in Japan, leading to a step toward an improved QOL in patients with designated intractable diseases and chronic diseases and their caregivers, as well as the realization of a more rich and healthy society.

Target of the Survey

During the survey period of four days from September 21, 2021, data on the treatment support being offered by medical institutions or pharmaceutical companies, the awareness and use of PSPs, and patient needs for PSPs, etc. were collected in an online questionnaire survey of 1,000 patients (male-female ratio: 626: 374) who were visiting hospitals/clinics, while receiving pharmaceutical care at home, for any of the 333 Japanese designated intractable diseases. There were 890 valid responses. By age group, the valid respondents were primarily those in their 50s (32.6%), followed by those in their 40s (27.3%) and those in their 60s (18.1%) (Figure 1).

The top five Japanese designated intractable diseases are Parkinson’s disease, ulcerative colitis, systemic lupus erythematosus, Crohn disease, and ossification of the posterior longitudinal ligament.²⁾ These top-five diseases also dominated the top ranks of the respondents’ diseases. Patients with these top-five designated intractable diseases, which in Japan account for 42% of all patients with designated intractable diseases, accounted for 49% of all the respondents in this survey. The respondents were primarily patients with ulcerative colitis (Figure 2).

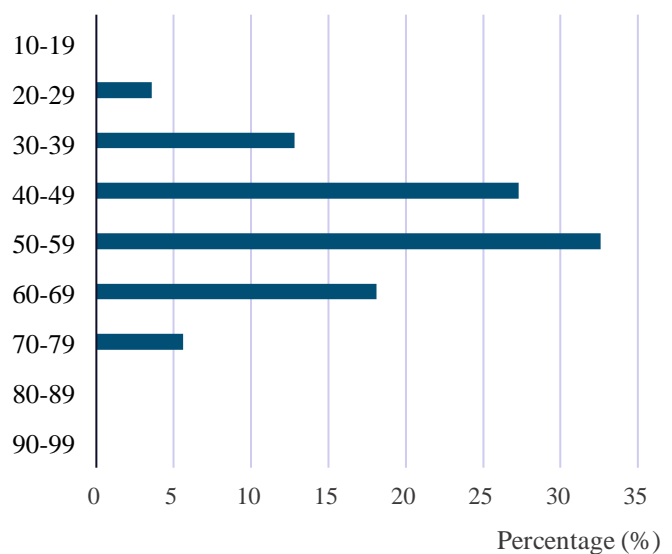


Figure 1. Distribution of respondents by age group (n=890)

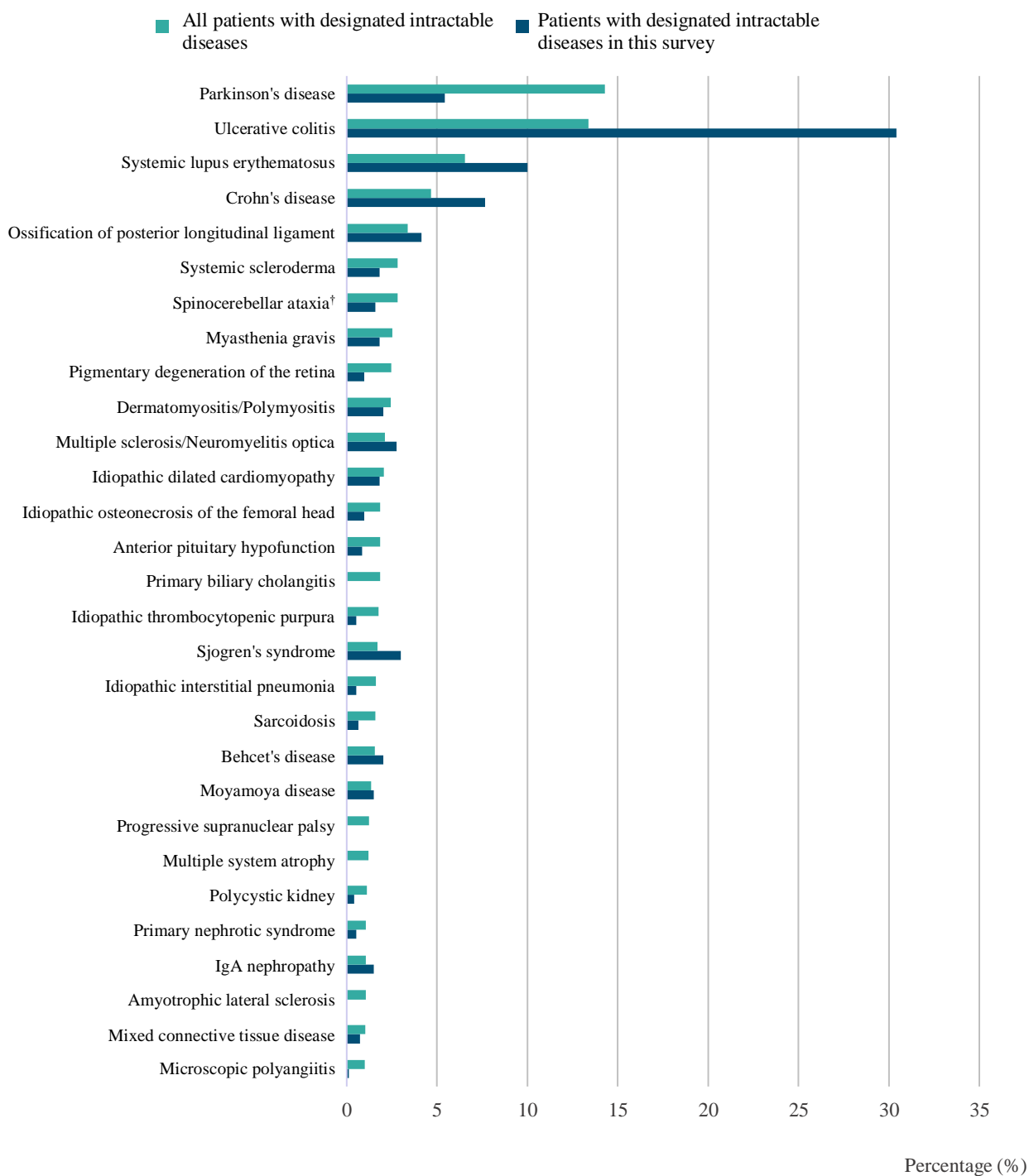


Figure 2. Distribution of the percentage of patients with designated intractable diseases in this survey among the top 25 diseases of all patients with designated intractable diseases*

Relationships with Health Care Providers from Patients' Perspectives

This survey included a question on the respondents' knowledge about their disease and current medications, as rated on a five-level scale ranging from 1 ("completely lacking") to 5 ("sufficient"). Of the 890 respondents, 46.6% gave positive ratings of 4 or higher, whereas 12.9% gave negative ratings of 2 or lower, including 1 ("completely lacking"). In relation to this, a question was asked about the level of their satisfaction with explanations about disease, medication, and medical care at home given by health care professionals including physicians. To this question, more than half of the respondents (59.4%) gave positive ratings of 4 or higher, including 5 ("satisfied"), whereas 10% gave negative ratings of 2 or lower, including 1 ("unsatisfied") (Figures 3 and 4).

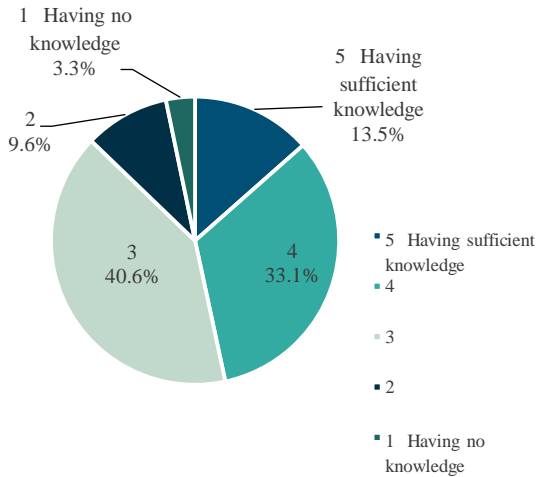


Figure 3. Knowledge of diseases and medications (n=890)

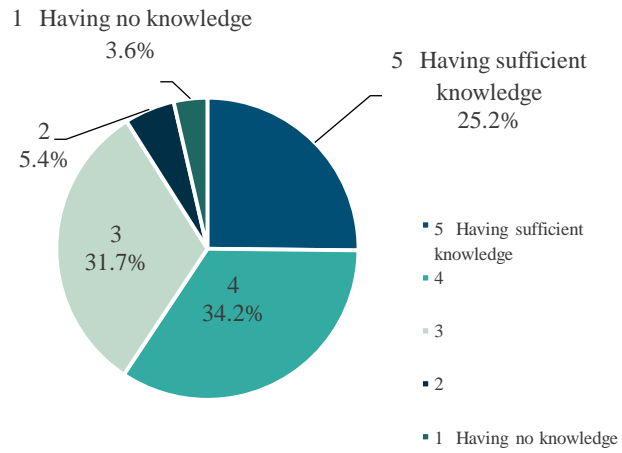


Figure 4. Satisfaction with explanations from healthcare providers (n=890)

Regarding the relationship with physicians, 68.9% gave positive ratings of 4 or higher, including 5 ("good"), whereas only 4.1% gave negative ratings of 2 or lower, including "bad." Thus, most responding patients felt that they had a good relationship with their physician. Conversely, to the question as to whether they consult their hospital or their regular physician regarding questions or worries about medications, their disease, and medical care at home, a certain percentage (18.5%) of patients answered "not often" or "never." Thus, it was revealed that more than half of the respondents, including those who rarely consult physicians, do not actively ask questions or consult physicians even when they have anxiety (Figures 5 and 6).

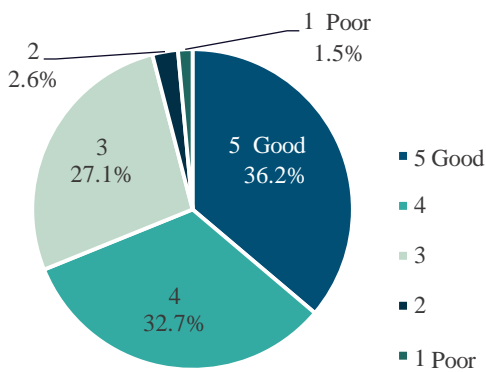


Figure 5. Relationship with physicians (n=890)

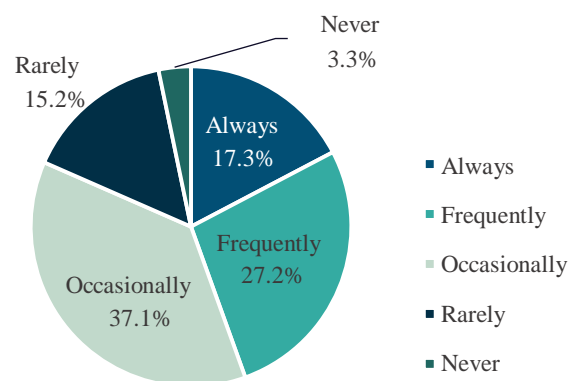


Figure 6. Frequency of asking questions to/consulting healthcare providers (n=890)

A question was also asked about the sources of information that the respondents use regarding their disease and medication, other than physicians and other healthcare professionals. Approximately 80% answered “the Internet,” whereas approximately 14% answered “nothing in particular.” The level of satisfaction with these information sources was rated positive as 4 or higher, including 5 (“satisfied”) by 48.7% of the respondents, whereas it was rated negative as 2 or lower, including 1 (“unsatisfied”), by 10.3% (Figures 7 and 8).

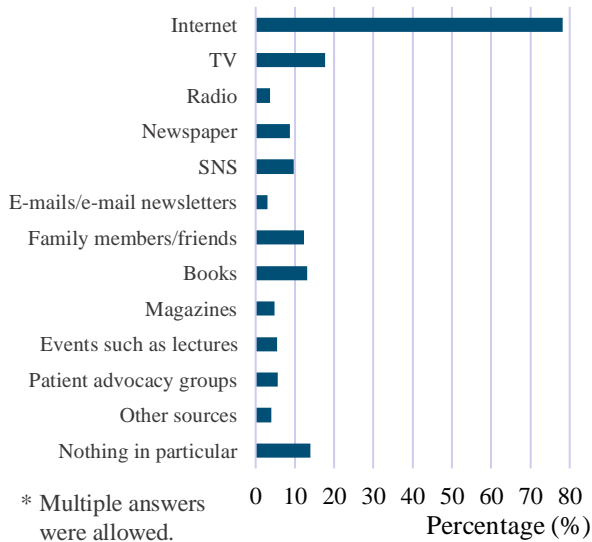


Figure 7. Sources of information on diseases

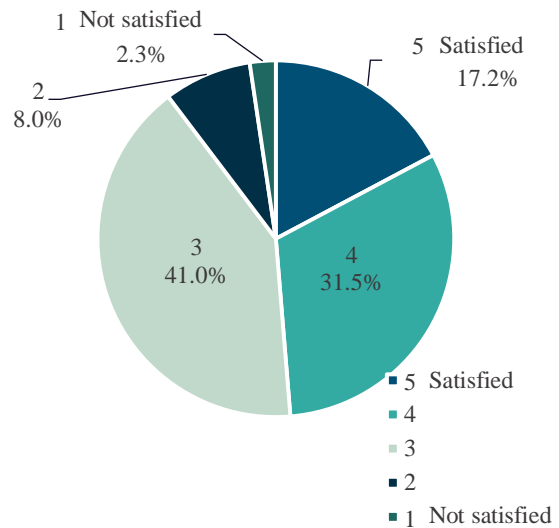


Figure 8. Satisfaction with sources of information

Proportions of Awareness and Use of Patient Support Programs for Designated Intractable Diseases and Trends

In this survey, 10.8% of the respondents answered that they “know of” PSPs, of whom 60.4% answered that they “have used” or “are using” PSPs. We cannot simply conclude that the awareness rate of PSPs is low because no PSP is available for some diseases. However, the rate is insufficiently high even with the proportion of respondents by disease taken into account, suggesting that raising the awareness of PSPs is an important challenge. Considering that appropriately 40% of the respondents do not use PSPs despite being aware of them, it is also important to provide services that meet patients’ needs (Figure 9).

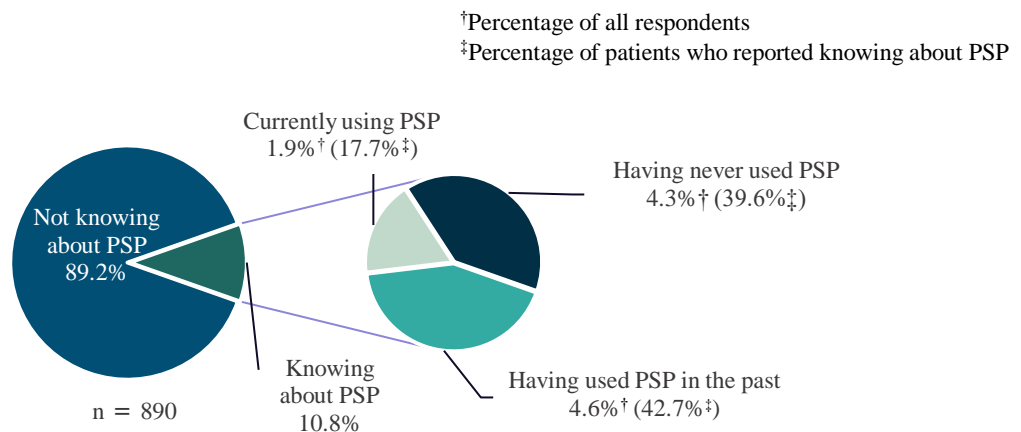


Figure 9. PSP recognition/utilization rate

The respondents were divided into the following three groups to analyze trends according to age groups: patients who have used PSPs (hereinafter, “users”), patients who know but have never used PSPs (hereinafter, “non-users”), and patients who do not know anything about PSPs (hereinafter, “those unaware”). The results showed that the users were primarily in their 30s. According to a comparison between the groups aware of PSPs (users and non-users) and the group of those unaware, users and non-users were primarily in their 40s, followed by 30s, whereas the group of those unaware were primarily in their 50s. The rate of unawareness by age group was more than 90% among those in their 50s, whereas it was 65.7% among those in their 20s and 78.9% in their 30s. Thus, younger generations exhibited lower rates of unawareness of PSPs. Meanwhile, regarding the PSP use rate among users and non-users, more than half of those in their 20s, 30s, and 40s have used PSPs after becoming aware of them. The reason why relatively younger generations more commonly use PSPs, as revealed by this survey, requires further analysis. However, raising the awareness of PSPs among a wide range of age groups and promoting the willingness to use PSPs of those in their 50s or older are also considered important in spreading PSPs (Figures 10 and 11).

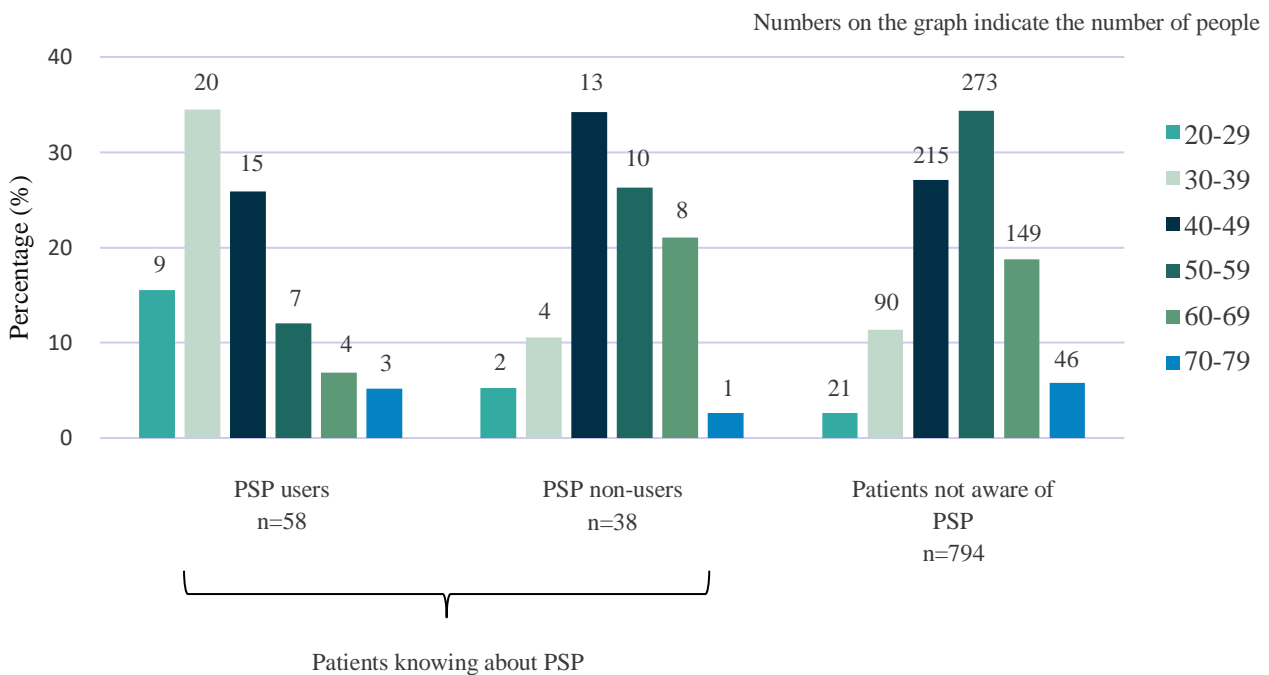


Figure 10. Distribution of PSP recognition/utilization by age group

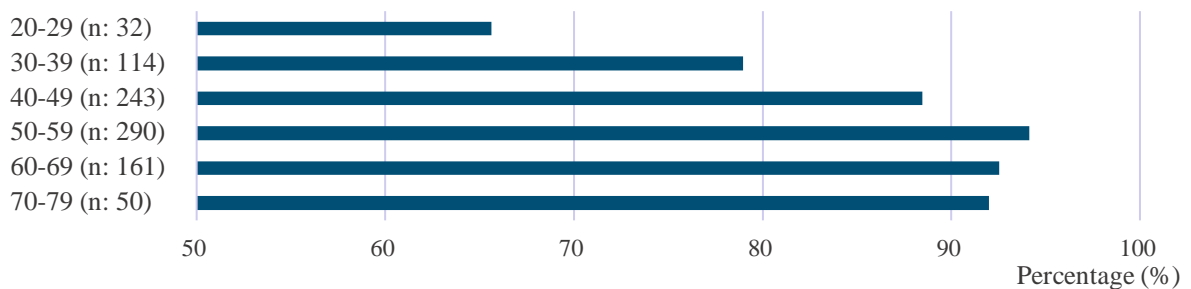


Figure 11. Percentage of patients not aware of PSP by age group

The reasons why the users and non-users did not use PSPs included those attributable to the lack of understanding of PSPs, such as “contents” and “how to join,” and those attributable to a failure to meet patients’ needs regarding usefulness, such as “not interested” and “unlikely useful.” With respect to these points, it is also important to appeal the importance of activities to make patients aware of PSPs and understand their needs (Figure 12).

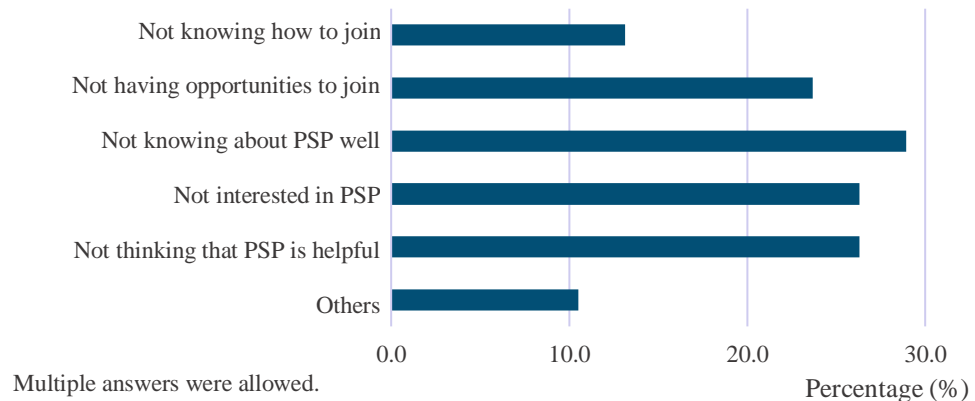


Figure 12. Reasons why patients do not join PSP (n=38)

Patient’s Needs and the Realities of Patient Support Services for Designated Intractable Diseases

In current survey, 40% or close to 40% of patients responded that they feel overwhelmed by the four components of treatment for incurable diseases that are "medication," "hospital visits," "application for beneficiary certificates," and "financial issues" (Figure 13). The services which the patients expect regarding PSPs were primarily the provision of information about “disease” and “treatment” and “information about drugs,” followed by “information about public systems.” Certain needs were also found for other items, such as information about “the sources of information about the disease and drugs,” “information about daily life,” and “counseling.” Meanwhile, 24.7% of all the patients answered “nothing in particular,” although none of the patients who had used PSPs answered so. Although the patients who answered “nothing in particular” might be satisfied with their current treatment environment, more active provision of information might allow us to meet the patients’ potential needs and thereby bring a higher QOL to them (Figure 14).

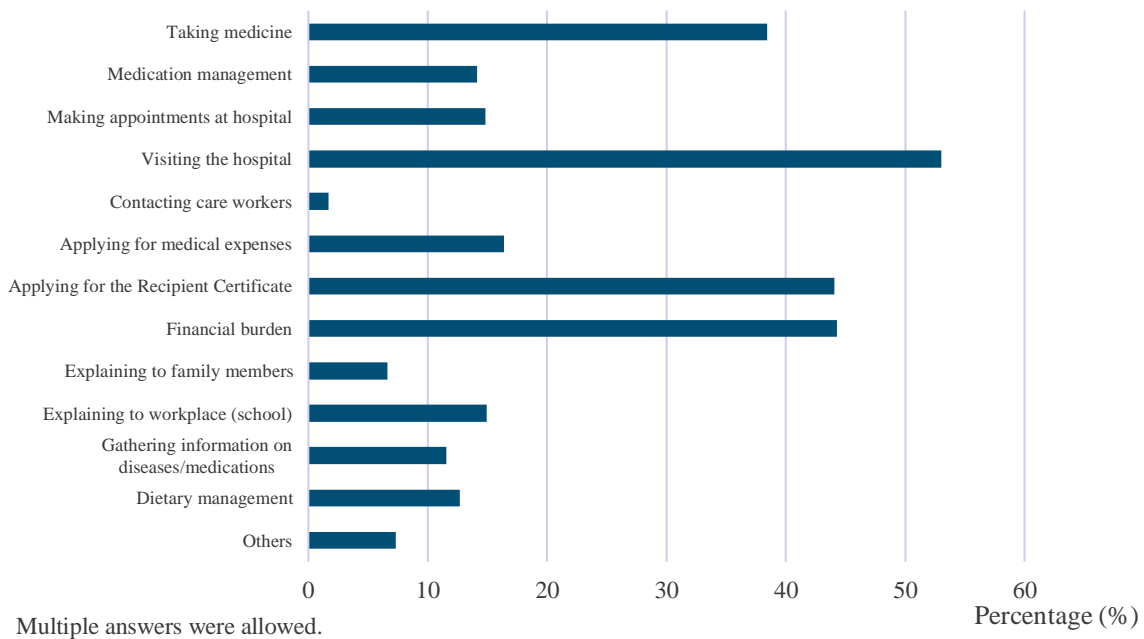
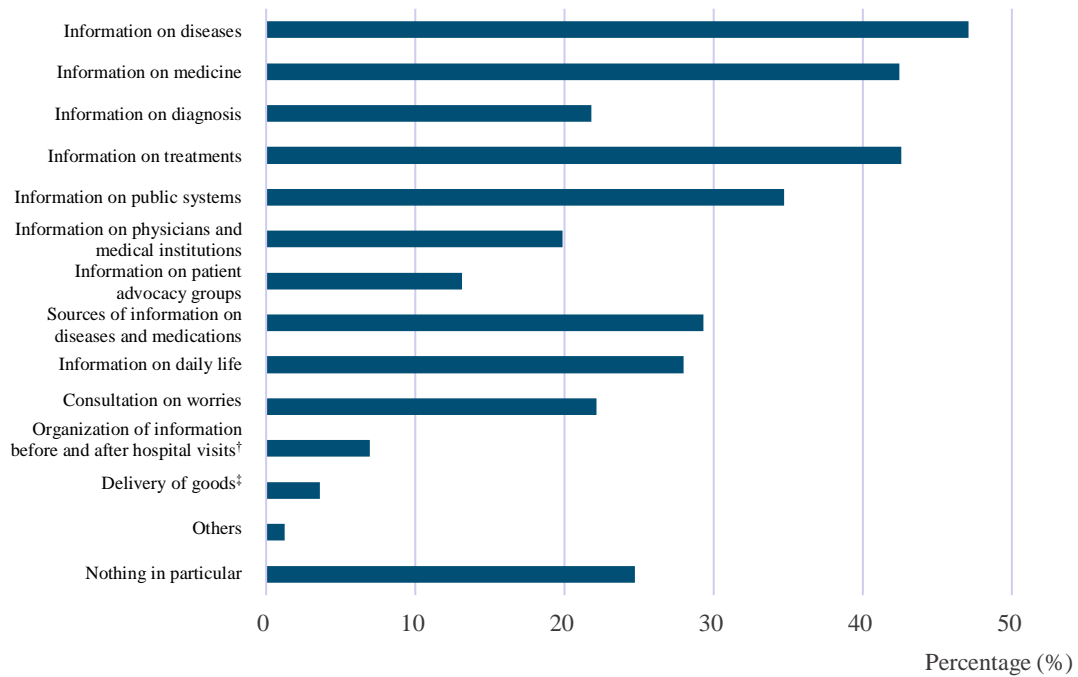


Figure 13. Matters that patients find burdensome in the treatment of intractable diseases



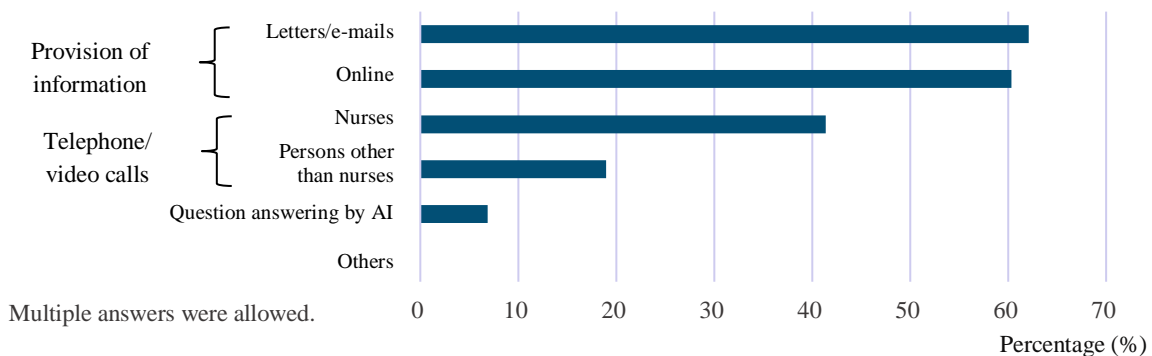
Multiple answers were allowed.

[†]Preparation prior to meeting with physicians to make surgical decisions, etc.

[‡]Cardboard box for needles, patient’s notebook, etc.

Figure 14. Services patients look for in PSP

The survey asked PSP users about the way services are delivered. Approximately 60% of users responded that they have received the support of information provision ‘via letter/e-mail’ or ‘online’. In addition, approximately 60% of users have received support through direct communication by telephone or video-conference, of which 41.4% and 19.0% involved a nurse or non-nurse, respectively. Although few in number, 6.9% of users have used an AI Q&A system (Figure 15).



Multiple answers were allowed.

Figure 15. Support in using PSP

When those who have no knowledge of PSP were asked by whom they would like to be provided such information other than a doctor, 46.0% of them answered ‘anyone’, which was the most common answer, followed by ‘a nurse’ (36.0%) and ‘a pharmacist’ (30.2%). On the other hand, the majority of PSP users or those who are aware of PSP want to be provided services by a qualified healthcare professional such as ‘a nurse’, ‘a pharmacist’ or ‘a physical therapist’, and there were 19.0% of PSP users who responded ‘anyone’ (Figure 16).

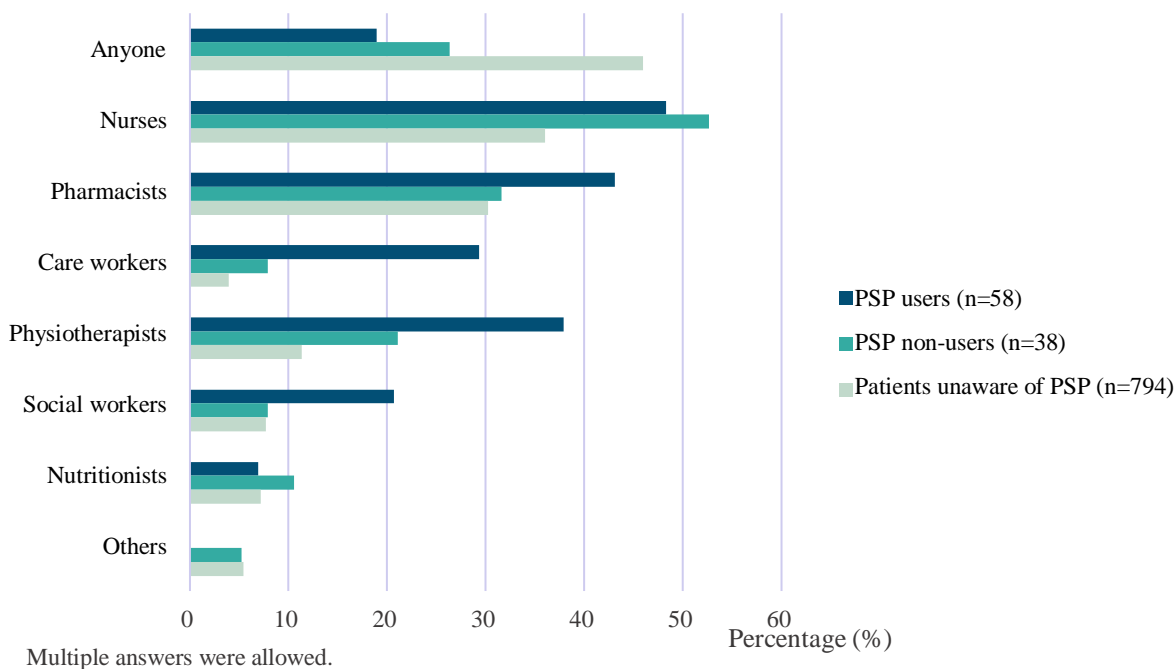


Figure 16. Service providers from whom patients seek answers on PSP

Additionally, the survey revealed that patients who want to receive information via e-mail accounted for approximately 70%, regardless of their knowledge of PSP, while those who want to receive services via social media accounted for more than 30%. However, although there was a minimal difference attributed to their knowledge of PSP, it was shown that PSP users seek service provision via telecom services such as telephone or video conference, suggesting that there may be a number of patients who experience its usefulness or convenience through actual utilization (Figure 17).

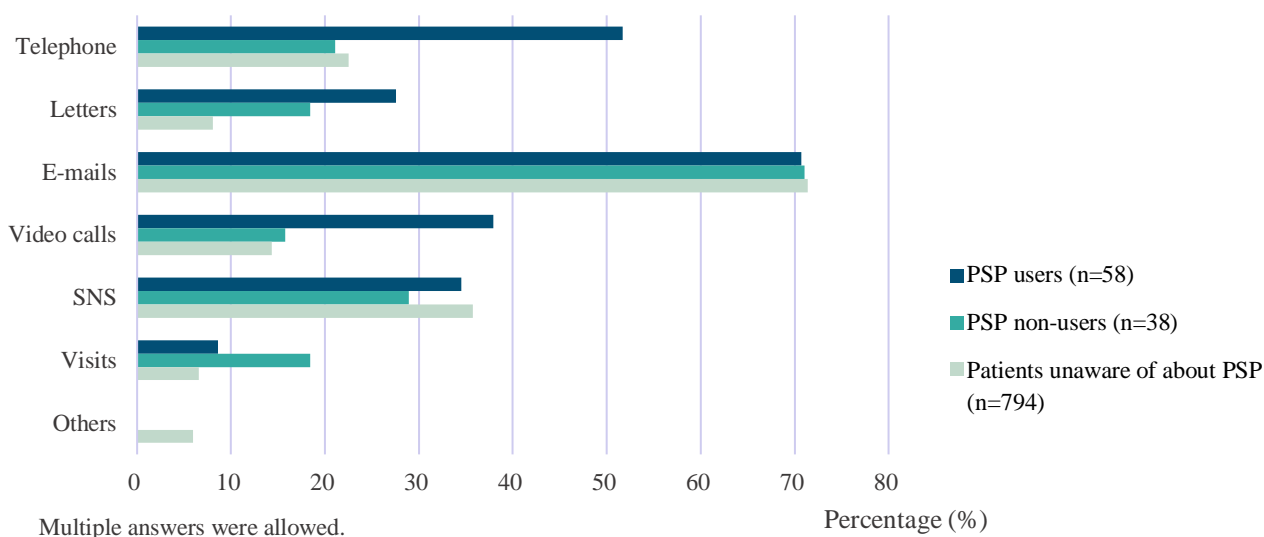


Figure 17. Medium that patients ask PSP to provide services through

Although few in number, there were also respondents who expect the following services with PSP (Table 1).

Table 1. Service contents required of PSP

| | |
|---------------|--|
| Information | Provision of the latest information |
| | Review of information provided by a healthcare professional |
| | Judgment/verification whether the information (on internet) is true or not |
| | Information on employers who have an understanding of diseases |
| | Information on similar symptoms and patients with such symptoms |
| Other support | Mental support |
| | Communication tools and exchange opportunities with other patients |
| | Explanation to their surroundings, including work colleagues and family members |
| | Support related to application filings or procedures <ul style="list-style-type: none"> • Support for cumbersome document preparations • Support leading to a reduction in burden of procedurally required multiple visits to the hospital or government office • Support leading to a reduction in burden of the required annual renewal procedure despite having an incurable disease |

Finally, the survey asked PSP users about the usefulness of PSP on a five-grade scale ranging from 1 of 'useless' to 5 of 'useful', resulting in 77.6% of PSP users who responded positively as 4 or higher including 'useful' and 3.4% of PSP users who responded negatively as 2 or lower including 'useless', suggesting that the patients with the designated intractable diseases who were included in the present survey have experienced the usefulness of PSP (Figure 18).

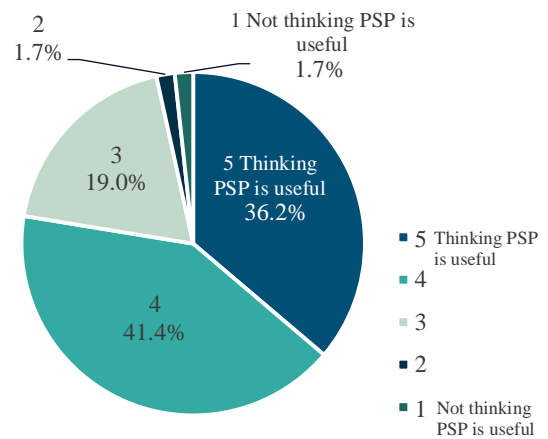


Figure 18. Satisfaction with PSP (n=60)

Conclusion

Today, there is a need for the patient-centric healthcare, PSP has been delivered in the US and Europe with the intention to improve patient adherence, raise awareness of diseases, support how to use specific pharmaceuticals, and support mental anxiety in patients and caregivers during treatment, while, also in Japan, there has been a situation where similar services are delivered. In Japan, even before PSP became available, medical institutions or pharmaceutical companies have provided various services from their own perspective in order to support patients and their families. These include providing information online, establishing consultation offices on medicines, providing information by distributing materials. This seems to have yielded some positive results, but the change in environment with an aim of achieving patient-centric healthcare, and the appearance of advanced therapies and medicinal products, suggest that such traditional ways have a limitation in meeting the needs of patients. The present survey also suggested the importance to deliver needed information in a timely manner through interactive information sharing, as the needs of patients with designated intractable diseases or chronic diseases and their family members (caregivers) undergo various changes depending on the severity or situation of their disease. Furthermore, the survey showed that, with advances in medical care and an increase in support by an expert, consultation with an expert on daily life and on treatment/medication of is needed by patients and their family members (caregivers), and services by an expert, such as a nurse or pharmacist, working at a consultation desk could lead to a high level of satisfaction for patients and their family members. It may be inevitable that PSP with such service contents and delivery systems will be disseminated and expanded to meet diversifying patients' needs.

Meanwhile, it is very important to draw the line between the services provided by PSP and medical practices, and misleading actions or inept support which may result in a loss of confidence in medical services in general, as well as in

PSP users. Therefore, for providing PSP, it is necessary to ensure thorough compliance with various regulations, and a full understanding of the individuals involved, for the significance and service contents of PSP.

Although PSP has just started in Japan, patients and their families who live with disease while receiving treatment may have various problems and require support (or could easily move toward solving problems with support) on a moment-to-moment basis, in terms of disease type or severity, restriction of physical activities, or complexity of dosing strategy. Various support services have been provided so far, but PSP is expected to be a program that could more closely listen to patients and their families, and provide needed support.

References cited

- 1) The number of holders of specific medical expense (designated intractable diseases) recipient certificates as of the end of FY 2020 according to the Japan Intractable Diseases Information Center
<https://www.nanbyou.or.jp/wp-content/uploads/2022/03/koufu20211.pdf>
- 2) Public Health Administration Report by the Health, Labour and Welfare Ministry (15 February 2022).
Reference: Public Health Administration Report cases of the fiscal year 2020, statistical table, annual report
<https://www.mhlw.go.jp/index.html>

Authors

Ryosuke Fujita
CMIC Ashfield Co., Ltd.
Director, Consulting Division, Medical Affairs Business Unit

Ikuko Igarashi
CMIC Ashfield Co., Ltd.
Director, Strategy Planning and Promotion Division, Medical Affairs Business Unit

Reiko Toyoda
CMIC Ashfield Co., Ltd.
Manager, Strategy Planning and Promotion Division, Medical Affairs Business Unit

Hikaru Mamiya
CMIC Ashfield Co., Ltd.
3rd group, MA/MSL Division, Medical Affairs Business Unit

Contact

Med Insights contact (medinsights-j@cmic.co.jp)

Operations

Med Insights is an information website operated by the Medical Affairs Business Unitⁱⁱ⁾ (hereinafter referred to as "the Unit") of CMIC Ashfield Co., Ltd.ⁱ⁾ (hereinafter referred to as "the Company"). The Unit offers comprehensive services to medical affairs divisions of pharmaceutical companies, focusing on various consulting services related to organization and medical strategy development, as well as medical affairs-specific training, management of MA Academy, patient support programmes, coaching services by field scientists, and dispatch of medical science liaisons (MSLs).

CMIC Ashfield Co., Ltd. is a joint venture between CMIC Group and Ashfield, healthcare companies operating globally.

CMIC Group is the first company in Japan to launch a Contract Research Organization (CRO, drug development support) business in 1992, and has responded to the various needs of its clients as a leading company. Now, CMIC group has established a system to provide services in all operations related to drug development beyond the framework of a CRO. CMIC group will contribute to the development of the medical and healthcare fields by building a business model that can create new value as well as meet customer expectations by making the most of our wealth of experience and expertise accumulated to date.

Ashfield Engage is part of the Inizio Group and operates in five areas: medical affairs, market access, commercial, patient solutions, and event experiences. Ashfield Engage helps its clients connect with all healthcare recipients and help them get knowledge, support, and medicine when and where they need them through its services. With a history of more than 20 years since its establishment in 1997, Ashfield Engage utilizes all channels to achieve the best and most effective communication for each situation as an expert in strategic engagement through programmes backed by experience and data.

Inizio is a leading global company providing professional services that help pharmaceutical and life science companies commercialize new therapies and support the drug lifecycle from development. Formed through the merger of Ashfield (UDG) and Huntsworth, Inizio offers a full suite of advisory, medical, marketing, communications, and patient/stakeholder engagement services.

- i) CMIC Ashfield Co., Ltd. (<https://www.cmic-ashfield.com>)
- ii) Medical Affairs Business Unit, CMIC Ashfield Co., Ltd. (<https://www.cmic-ashfield.com/service/non-commercial/>)

Disclaimer

This document is intended to provide information to the public and includes the authors' consideration at the time of writing based on their research and business experience. Neither CMIC Ashfield Co., Ltd. nor CMIC Group is providing professional advice or services with this document.

No representation, warranty, or assurance, express or implied, is made as to the accuracy, completeness, suitability, or reliability of the information contained in this document. In no event will the Company be liable, directly or indirectly, for any loss or damage whatsoever which may arise from reliance on this document.

Except as permitted by the Copyright Act, any duplication, public transmission, alteration, modification, reprinting, or other use of this document without our permission is prohibited by the Copyright Act. If you wish to reprint, duplicate, or otherwise use this document, please contact or consult with us in advance.

