HGPI Health and Global Policy Institute

難病・希少疾患プロジェクト

「難病の日」シンポジウム
「患者・市民の視点から考えるこれからの難病対策」報告書

Intractable Disease and Rare Disease Project

Intractable Disease Day Symposium:
“Future Measures for Intractable Diseases from the Perspectives of Patients and Citizens” Report

2024年5月23日（木）
Thursday, May 23, 2024

開催概要

日本医療政策機構（HGPI）は2004年に設立され、「市民主体の医療政策の実現」を掲げる非営利・独立・超党派の政策シンクタンクとして活動している。感染症や非感染性疾患といった従来の医療政策課題にも取り組んでいるほか、女性の健康や子どもの健康、プラネタリーヘルスといったこれまでの医療政策の枠組みに留まらない、広範な社会課題にも焦点を当て、市民社会から政策の選択肢を提示することを目指している。

難病は数多ある医療政策課題の中でも、特に重要な課題の1つである。希少疾患と合わせて議論されることが多いが、特に難病の場合はその発生機序が明らかでなく、明確な治療法が確立されてない、また長期の療養が必要になるなど、患者の心身の負担が大きくなるとされている。日本では、指定難病とされる疾患は今日現在341に上り、患者数は100万人を超えるとされる。政府も1972年の難病対策要綱を皮切りに、「研究開発の推進」「医療提供体制の整備」「患者支援（主に医療費助成）」という形で難病対策を進めてきた。また1998年には、難病医療連絡協議会、難病支援センターが各都道府県に設置され、患者の生活環境の整備や就労支援など、福祉制度との連携も進んでいる。また介護保険制度においても難病を原因とする要介護への対応も可能となっている。2014年には「難病の患者に対する医療等に関する法律（難病法）」が制定され、医療の推進をはじめ、社会環境の整備も含めた方向性を打ち出している。

一方で依然として難病対策の課題も様々な指摘がなされている。医療提供体制の面では、必要な情報が十分に行き届いていない情報格差や、診断までの時間が依然として長い状況（診断ラグ）、さらには医療の地域格差、小児医療から成人医療への連携などが挙げられる。また研究開発においては、各疾患に対する基礎的な研究のさらなる推進に向けたデータ収集体制の構築や、治療薬の開発に向けて患者ニーズに基づく研究の推進に向けた研究への患者参画（PPI: Patient and Public Involvement）などが期待されている。

本シンポジウムでは、これからの難病対策のあり方についてマルチステークホルダーによる議論を通じて、今後の論点を整理すると共に、それらを社会に広く発信することを目的とした。

日時： 2024年5月23日（木）15:00-17:00
形式： ハイブリッド（対面・オンライン（Zoomウェビナー））
会場： 大手町フィナンシャルシティ グランキューブ3階 イベントフィールド

プログラム：（敬称略・順不同、肩書は開催当時）

15:00-15:05 **開会のお言葉：「難病の日」を迎えて**

辻 邦夫（一般社団法人 日本難病・疾病団体協議会 常務理事）

15:05-15:20 **プレゼンテーション1「市民社会の視点から考える難病対策のこれまでとこれから」**

西村 由希子（特定非営利活動法人ASrid 理事長）

15:20-15:35 **プレゼンテーション2「日本の難病対策について」**

横田 正明（厚生労働省健康・生活衛生局 難病対策課 課長補佐）

15:40-15:55 **プレゼンテーション3「AMEDにおける希少難治性疾患への取り組み」**

中島 唯善（国立研究開発法人日本医療研究開発機構（AMED）
創薬事業部 創薬企画・評価課 調査役／AMEDプログラムオフィサー）

15:55-16:10 **プレゼンテーション4「自治体における難病対策」**

津島 志津子（神奈川県健康医療局 保健医療部がん・疾病対策課 課長）

16:10-16:25 **プレゼンテーション5「患者・市民と共につくる研究」**

古結 敦士（大阪大学大学院医学系研究科 医の倫理と公共政策学分野 助教）

16:30-17:00 **パネルディスカッション「患者・市民の視点から考えるこれからの難病対策」**

大坪 恵太（一般社団法人 日本難病・疾病団体協議会 事務局長）
古結 敦士
中島 唯善
西村 由希子
横田 正明

モデレーター

栗田 駿一郎（日本医療政策機構 シニアマネージャー）

Overview

Health and Global Policy Institute (HGPI) is a non-profit, independent, non-partisan policy think tank established in 2004 with the mission of achieving citizen-centered health policy. In addition to providing policy options to society for conventional health policy issues like infectious diseases and non-communicable diseases (NCDs), HGPI is also taking action on various issues that impact society but were previously viewed as separate from the health policy framework, such as women's health, children's health, and planetary health.

Although health policy spans a wide variety of topics, intractable diseases are one of the most important. While they are often discussed alongside rare diseases, certain aspects of intractable diseases place particularly heavy physical and psychological burdens on patients. We have yet to identify the underlying mechanisms or establish clear treatment methods for intractable diseases. They also require patients to undergo long-term treatment. Japan currently designates 341 conditions as "intractable diseases" and they are estimated to affect over 1 million people.

Starting the compilation of the "Outline of Intractable Disease Measures" in 1972, the Government of Japan has been advancing measures for intractable diseases through the promotion of R&D, improving the healthcare provision system, and supporting patients, mainly through the subsidization of care. After the formation of the Liaison Council for Intractable Disease Treatment and establishment of Intractable Disease Support Centers in each prefecture in 1998, joint efforts with the welfare system have been underway to improve living environments and provide employment support for people living with intractable diseases. The Long-Term Care Insurance System has made it possible to provide long-term care to people who require it due to an intractable disease, and a direction for intractable disease measures that include promoting healthcare and improving the social environment was set by the enactment of the Act on Medical Care for Patients with Intractable Diseases in 2014.

However, measures for intractable diseases face a number of lingering issues. For the healthcare provision system, these include information disparities, in which people do not receive enough of the information they need; long diagnostic delays or diagnosis lag; regional healthcare disparities; and collaboration spanning pediatric healthcare to adult healthcare. There are also a number of actions that should be taken for R&D. For example, we hope to see the establishment of a data aggregation system to further basic research on each disease as well as the promotion of Patient and Public Involvement (PPI) to encourage research that reflects patients' needs in the development of therapeutics.

At this symposium, we held a multi-stakeholder discussion on the nature of intractable disease control to identify issues to address in the future and disseminate those issues to greater society.

Date & Time: Thursday, May 23, 2024; 15:00-17:00 JST

Format: Hybrid (In-Person and Online (Zoom Webinars))

Venue: Event Field, Grand Cube 3F, Otemachi Financial City, Global Business Hub Tokyo

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Program: (In no particular order; titles omitted. Please note that positions listed are current as of the day of the meeting.)

15:00-15:05 **Opening Remarks: Marking the Occasion for Intractable Disease Day**

Kunio Tsuji (Standing Director, Japan Patients Association (JPA))

15:05-15:20 **Presentation 1: The Past and Future of Measures for Intractable Diseases From the Viewpoint of Civil Society**

Yukiko Nishimura (President, NPO ASrid)

15:20-15:35 **Presentation 2: Measures for Intractable Diseases in Japan**

Masaaki Yokota (Deputy Director, Intractable Disease Control Division, Public Health Bureau, Ministry of Health, Labour and Welfare (MHLW))

15:40-15:55 **Presentation 3: Current Initiatives in Intractable Diseases from AMED**

Tadayoshi Nakashima (Research Officer, Intractable disease/Rare disease, Rare/Intractable Disease Project, Division of Strategic Planning and Evaluation, Department of Innovative Drug Discovery and Development, The Japan Agency for Medical Research and Development (AMED))

15:55-16:10 **Presentation 4: Measures for Intractable Disease in Local Governments**

Shizuko Tsushima (Director, Cancer and Disease Control Division, Healthcare and Medical Services Department, Health and Medical Services Bureau, Kanagawa Prefectural Government)

16:10-16:25 **Presentation 5: Co-creating Research with Patients and Citizens**

Atsushi Kogetsu (Assistant Professor, Department of Biomedical Ethics and Public Policy, Graduate School of Medicine, Osaka University)

16:30-17:00 **Panel Discussion: The Future of Intractable Disease Control from the Perspectives of Patients and Citizens**

Keita Otubo (Secretary General, Japan Patients Association (JPA))
Atsushi Kogetsu
Tadayoshi Nakashima
Yukiko Nishimura
Masaaki Yokota

Moderator

Shunichiro Kurita (Senior Manager, HGPI)

開会のお言葉：「難病の日」を迎えて

辻 邦夫（一般社団法人 日本難病・疾病団体協議会 常務理事）

日本難病・疾病団体協議会（JPA: Japan Patients Association）は、2014年5月23日に「難病の患者に対する医療等に関する法律」（難病法）が成立したことを記念し、毎年5月23日を「難病の日」に登録した。本年は、難病法成立から10年の節目にあたる。

2022年3月末現在、国が定める「指定難病」の医療費助成を受けている患者数は105万人、小児慢性特定疾病の患者数は10.5万人いるとされている。これを基に推計すると、国民の100人に1人は何らかの難病を抱えていることになる。

治療・研究の進歩により、健常の人と同じように生活している難病患者が増えている一方、再発や再燃、あるいは病気の進行によって、例えば就労継続の問題に直面するケースも起きている。また、難病患者であっても障害者手帳を保有している人の割合は3割に留まることから、就職活動の際に自分が難病患者であると会社に伝えることを躊躇してしまうという声がよく聞かれる。

厚生労働大臣は難病基本方針において、難病の患者が難病であることを安心して開示し、治療と就労を両立できる環境を整備することとしているが、実際には難しい現状がある。また、医療提供体制や相談支援体制の地域格差、医療的ケア児の就学、小児期から成人期への移行（トランジション）、さらには障害年金や障害者福祉に当事者が関われない等の問題もある。

日本が成熟した社会として、国民の誰しもが発症する可能性のある難病の患者、家族を支えていくために、難病法は制定された。これまで医療政策はもとよりさまざまな社会課題に焦点を当てた政策提言を発信している日本医療政策機構のプロジェクトに、難病のプロジェクトが加わったことに大きな期待を寄せている。本日のシンポジウムが実り多いものとなり、当事者をはじめ多様なステークホルダーが参画することによって、よりよい提言となることを願っている。

プレゼンテーション1「市民社会の視点から考える難病対策のこれまでとこれから」

西村 由希子（特定非営利活動法人ASrid 理事長）

1972年10月に難病対策要綱が策定された当時、「難病」といえば希少疾患（患者数が少なく完治の難しい疾患）であった。その42年後に制定された難病法における「難病」という言葉には、希少疾患、難治性疾患、長期慢性疾患の3つが含まれている。2014年に成立した難病の患者に対する医療等に関する法律（難病法）は、関係者が待ち望んだ法律ではあるものの、「難病」はすでに社会通念的な言葉となっているため、法律内で使われている「難病」という言葉は、患者・家族がイメージする「難病」とは若干の乖離がある。

2022年12月10日に成立した難病法改正によって始まる新たな難病対策について、当事者・家族にアンケート調査（2023年9月30日～11月8日）を実施した結果、良かった点として「重症化時点までの遡り請求」「登録者証の発行」などが挙げられた一方、「対象者や認定の見直し」「助成費用の拡充」などさまざまな課題も寄せられた。

昨今、難病領域と他領域との連携、一般市民との連携が進んでいる。
私たちは、臨床試験に関する正しい情報がわかりやすく届いているかを関係者「みんな」で協議し、要望を「適切に」伝えるため、「臨床試験にみんながアクセスしやすい社会を創る会」を2023年6月に発足した。発起人は、がん領域と希少・難治性疾患領域の協議会や中間機関、研究者や医師が務め、オブザーバーとして厚生労働省および保健医療科学院が、事務局として製薬協が関わっている。このようなメンバーと共に、わかりやすくアクセスしやすいデータベースの構築に向けた要望書を政府に対して提出したところである。

一般への啓発活動として、2008年に欧州16カ所で始まった世界希少・難治性疾患の日（RDD: Rare Disease Day）を挙げる。RDDは、希少・難治性疾患の患者・家族の生活の質の向上を目指した本領域で世界最大の社会啓発イベントである（毎年2月末日に開催）。日本では2010年から始まり、15周年を迎えたRDD2024は全国75カ所で開催された。

RDD2024の公認開催主催者は、さまざまな疾患領域の患者会や患者支援組織、病院などの医療サイドに留まらず、図書館、中学・高校、商店街など多岐にわたる。希少疾患や難病について多くの人々に知ってもらい、一緒に考えたいという思いから、多様な主体が積極的に情報を発信しているのが特徴である。

私たちはこれからも、“患者の”ための情報を、“患者と”ともに考え、“みんなが”一緒に伝えていく。
「私たちのことを私たち抜きで決めないで」という言葉があるが、難病領域にも当てはまる。難病領域は幅広く、全てを包含するのは難しいかもしれない。しかし、対象となる患者がチームの一員に加わり、一緒に考えていく必要がある。それを一般社会の人々にみんなで一緒に伝え、アクションを起こしていくことが重要である。

横田 正明（厚生労働省健康・生活衛生局 難病対策課 課長補佐）

改正難病法・改正児童福祉法のポイント
難病患者及び小児慢性特定疾病児童等に対する適切な医療の充実や療養生活支援の強化、指定難病及び小児慢性特定疾病のデータベースに関する規定の整備等が盛り込まれた改正難病法・改正児童福祉法が、昨年から本年にかけて段階的に施行されている。

この難病・小慢対策の見直しは、2021年7月に、難病や小児慢性特定疾病の患者団体の方等も構成員となっている厚生労働省の審議会においてまとめられた意見書を踏まえ、行われたものである。

主な法改正内容として、まず、2023年10月からは、医療費助成開始時期が申請時点から重症化時点とし、その遡りは申請日から最長3カ月まで可能となった。また、地域における支援体制の強化として、難病相談支援センターと福祉・就労支援関係者との連携が法律上規定されるとともに、小児慢性特定疾病対策地域協議会の法定化や難病と小慢の地域協議会間の連携努力義務等が施行された。

さらに、本年4月からは、福祉、就労等の支援を円滑に利用できるようにするため、指定難病に罹患していること等を証明する「登録者証」を発行する事業が創設された。また、難病・小慢データベースに関しては、本人が特定されないよう匿名加工したデータを製薬会社等にも提供可能となった。これにより、例えば製薬企業の研究開発においては、特定の患者群に関する疫学情報の整理・把握や、個別の患者の新たなデータの収集・患者へのアプローチに向けた情報の把握・分析等に活用できる可能性があり、ぜひ、創薬に向けて活用いただきたい。

医療DXの推進と今後の難病対策の方向性
保健・医療・介護の情報の利活用を推進し、個人の健康増進に寄与するとともに、医療現場等における業務効率化の促進、より効率的・効果的な医療等のサービス提供を行っていくことは重要であり、医療DXの実現に向けて、2023年6月に「医療DXの推進に関する工程表」がとりまとめられた。この工程表に基づく医療DXの取組を通じて、難病や小児慢性特定疾病の方を取り巻く環境を改善していきたいと考えている。

例えば、マイナンバーカードと健康保険証の一体化の取組が進められているが、医療費助成等に係る情報連携システム（PMH: Public Medical Hub）を整備し、マイナンバーカードを医療費助成の受給者証として利用できるようにする。これにより、難病患者等は、紙の受給者証の持参の手間が軽減し、紛失リスクや持参忘れ、再来院を防止することができる。医療機関にとっても、受給者証情報の手動入力負荷を削減しつつ最新の医療費助成受給資格を確認可能となり、医療費助成資格の確認事務コスト削減につながる。

また、デジタル技術を最大限に活用し、医療機関等における負担の極小化をめざし、「診療報酬改定DX」の取組を進めている。共通算定モジュール（標準型レセコン）の開発や公費・地単公費の医療費助成情報マスタの作成等を進めており、診療報酬改定DXの仕組みが全医療機関等に普及することにより、地単公費の現物給付化や医療機関等またぎの高額療養費も計算できる（償還払い不要）ほか、公費・地単公費に係る紙の上限額管理票を電子化することもできる。これは、医療機関等の負担軽減のほか、難病患者等にもメリットがある。

さらに、難病等に関する有効な治療法や創薬・医療機器の開発等の推進は重要であり、患者本人の権利利益を適切に保護しながら、研究者や製薬企業等が医療等情報をビッグデータとして分析できるようにし、医学・医療のイノベーションの成果を国民・患者に還元できるような環境整備を行っていく。例えば、相当の公益性がある場合に仮名化情報の利用・提供を可能とすることや、公的データベースを一元的かつ安全に利用・解析でき、セキュリティ保護が行われたVisiting解析環境を構築することなど、医療等情報の二次利用に関する検討を現在進めている。

最後に
冒頭に御説明した通り、2022年に改正難病法・改正児童福祉法が成立し、関係者の準備や御協力もあり、2023年10月と本年4月に、それぞれ改正法の内容を円滑に施行することができた。ただ、まだ取り組むべきことがあると考えており、本日は医療DXの取組に関する検討状況を御紹介したが、引き続き、関係者の御意見を伺いながら、難病患者や小児慢性特定疾病児童等のために、一つずつ取組を前に進めていきたい。

中島 唯善（国立研究開発法人日本医療研究開発機構（AMED）
創薬事業部 創薬企画・評価課 調査役／AMEDプログラムオフィサー）

難病対策研究の支援体制
日本医療研究開発機構（AMED: Japan Agency for Medical Research and Development）では、「第2期医療分野研究開発推進計画」に基づき、主要7疾患（がん、生活習慣病、精神・神経疾患、老年医学・認知症、難病、成育、感染症）に関して、医療分野の基礎から実用化までのプロセスや種別に6つの統合プロジェクトが横断的に対応できる体制を整えている。

難治性疾患実用化研究事業（以下、難治事業）では、そのうち5つの統合プロジェクト（医薬品、医療機器・ヘルスケア、再生・細胞医療・遺伝子治療、ゲノム・データ基礎プロジェクト、疾患基礎研究）にそれぞれ担当者を配置している。

難治事業は、指定難病に限らず「希少性」「原因不明」「効果的な治療方法未確立」「生活面への長期にわたる支障」の4要件を満たす希少難治性疾患を対象としてる。病因・病態の解明、画期的な診断・治療・予防法の開発による疾患の克服を目指し、毎年約200の課題に対する研究を支援している。

希少難病領域における研究の特徴
患者数が少ないために事業化が困難であり（低市場性）、企業の協力が得られにくい。承認申請に必要な症例数も集積しにくく、開発難度は高い。また、病態が不明なため開発ターゲットの特定が難しく、評価手法も未確立なものがある。

さらに従来の治療法で効果が得られない難病にはアンメットメディカルニーズが多く、核酸医薬、遺伝子治療、再生医療など、最新の科学技術に挑戦する課題が多いのも特徴といえる。また代替治療がないため、ベネフィット・リスク評価が他疾患とは異なる場合がある。難治事業において支援する研究者の多くはアカデミアであり、指定難病は341、難病は8,000以上となっている。

2022年3月には、希少難治性疾患における日米欧中の規制対応、開発企業や周辺環境の現状及び動向調査を実施し、その結果を新規公募設計に活用するため、超希少疾患、レジストリ（RWD）活用、個別化医療に取り組む研究者の支援を開始した。難病プラットフォームによる難病研究データの活用促進や未診断疾患イニシアチブ（IRUD: Initiative on Rare and Undiagnosed Diseases）の取り組みも進めている。

難治事業における最近の成果として、デュシェンヌ型筋ジストロフィーに対する核酸医薬の「ビルトラルセン」の薬事承認、全身性強皮症や難治性天疱瘡に対する「リツキシマブ」の薬事承認などに加え、2024年には難治性脈管腫瘍及び難治性脈管奇形に対する「シロリムス」を含む3剤の薬事承認をすでに取得している。

国際目標に則ったAMEDの活動
患者数の少ない希少難病では、国際連携も重要である。AMEDは国際希少疾患研究コンソーシアム（IRDiRC: International Rare Diseases Research Consortium）のメンバーとして参加し、確定診断、新規治療法、評価手法の確立に向けて活動している。その成果として、希少疾患の表現型の効率的情報共有方法（用語の統一、データシェアリング等）に関する国際的な状況を把握し、IRUDの立ち上げに生かすことができた。さらに未診断疾患の診断精度の向上等、IRDiRCでの検討成果を取り込んだことで診断率44％を達成した。

研究への患者・市民参画（PPI: Patient and Public Involvement）といった社会共創の推進も、難病領域においてとくに大切である。AMEDでは、研究や臨床試験実施計画書（プロトコル）作成の段階から患者さんと十分相談をすることを研究者に求めている。

津島 志津子（神奈川県健康医療局 保健医療部がん・疾病対策課 課長）

神奈川県における難病対策の現状
神奈川県において指定難病と診断され、一定の基準を満たした方は2024年3月 31 日時点で県全体で約6万人といわれ、2024年4月1日時点の対象疾患は14疾患群、341疾病となっている。

難病患者の支援体制では、かながわ難病相談・支援センターを設置し、地域で生活する患者等の日常生活における相談・支援、地域交流活動の促進及び就労支援を実施している。運営者は、神経難病で治療実績のある国立病院機構箱根病院（平成29年度より県及び3政令市による共同運営委託）である。2023年度の相談実績は1,239件で、療養生活に関する各種情報、医療機関や制度の案内、就労、患者会やピアサポートに関する相談が多い。

また、地域の保健所でも相談を受け付けている。保健所や市町村は難病対策地域協議会、ハローワーク（就労相談窓口）や産業保健総合支援センター（両立支援）等を通じて情報共有・連携し、地域のつながりを確保している。

難病医療提供体制については、難病医療連携拠点病院（4大学病院）、8つの医療圏域ごとの難病医療支援病院（29病院）を指定しており、その他一般病院や診療所を含めて相互に患者受け入れ調整や研修の実施、医療情報の提供体制を整備している。さらにレスパイト（病院独自を除く）を希望する場合、9病院で受け入れ可能となっている。2023年度には難病医療支援病院を新たに３病院指定し、地域偏在が改善した。さらにこれらに施設や保健所・市町村等を中心に相談支援体制も整備している。

難病対策協議会で捉えている今後の課題
「小児発症難病患者への切れ目ない医療提供体制」「難病情報連携センターと拠点病院間の情報共有の在り方について」「難病患者の災害時対策」などを始めとした6つの課題が挙がっている。

その中でも「小児発症難病患者への切れ目ない医療提供体制」に関しては、すでに検討が行われている移行期医療に係る体制構築とともに協議を進めている。2023年度に行われた第1回協議会では、入学や就職、妊娠出産時等の患者支援の必要性、医療機関間の患者に関する連携不足、小児慢性特定疾病から指定難病に移行できない疾患の存在や課題に対する認識不足、小児慢性特定疾病や難病に関する庁内関係部署の連携強化の必要性などが指摘された。

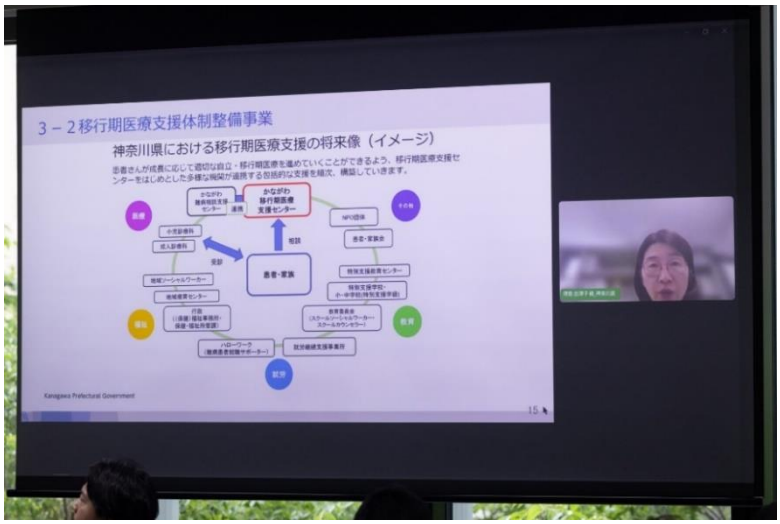
神奈川県では、小児慢性特定疾病児童等自立支援事業（地域の社会資源を活用するとともに利用者の環境等に応じた支援を行う事業）や移行期医療支援体制整備事業（小児期及び成人期をそれぞれ担当する医療従事者間の連携など支援体制の整備や、自身の疾病等の理解を深めるなどの自律（自立）支援の実施により、移行期医療支援体制の整備を目的とする事業）を実施している。

小児慢性特定疾病の患者さんが思春期、成人期を迎える時期には、小児診療科から成人診療科へ移行し、適切な医療を受けるべきである。ライフステージに応じて適切な医療の提供と自立支援が行われるように、小児期から成人期への一体的な支援体制の構築が必要と考えている。

切れ目ない医療提供体制の実現に向けた取り組み
移行期医療支援体制整備事業では、国立病院機構箱根病院に委託し、かながわ移行期医療支援センターを設置している。主な取組内容は、移行期医療支援コーディネーターによる相談支援（自立に向けた助言、各種制度の案内等）、小児・成人診療科の連携のための移行期医療体制の構築・整備（地域、領域別で対応可能な成人診療医療機関情報の収集・公表等）であり、相談実績も徐々に伸びている。

また、県内医療機関や行政機関を対象とした医療講演会を年1回開催（現地開催及びオンライン開催）しており、広く意見を聞く機会としている。

神奈川県における移行期医療支援の将来像として、患者さんが成長に応じて適切な自立・移行期医療を進めていくことができるよう、移行期医療支援センターをはじめとした多様な機関が連携する包括的な支援を順次構築していく計画である。



古結 敦士（大阪大学大学院医学系研究科 医の倫理と公共政策学分野 助教）

患者・市民の医療・研究・政策参画への背景
科学技術の進展や医療の高度化に伴い、「専門家主導の医療・研究・政策」と「患者さんの困難やニーズ」の間にギャップが生じるという問題が認識されるようになった。そうした中で、ここ20年ほど、医療・健康分野において医療実践や医学研究への患者・市民参画（PPI: Patient and Public Involvement）が広がりつつある。その背景には、市民運動・患者の主体的な活動の高まり、国の施策としての推進がある。医療保健福祉領域における市民参画を支援することを目的にイギリスの国立衛生研究所により設立されたINVOLVEは、2017年にPPIを「患者・市民のために、または患者・市民について研究が行われることではなく、患者・市民と共に、または患者・市民によって研究が行われること」と定義している。

日本では2000年代後半、とくにがん領域においてPPIの動きが広がった。2014年には、RUDY JAPAN（希少疾患を対象とした患者参加型研究）の構想開始、J-RARE公開（希少疾患レジストリ運営に患者会関係者が参画）、IPS 細胞の臨床研究に関するPPIの取り組み（武藤ら）など、研究へのPPIが進展する。その後、AMEDをはじめ国の組織によるPPI推進の動きが見られるようになった。

近年、政策形成の際に何らかの根拠（エビデンス）を踏まえて政策を立案し実装することが重要視されている。同時に、その過程に当事者が関与することの重要性が認識されている。その医療・健康分野における具体例として、研究の優先順位設定への患者参画が注目されている。一方、先行事例はあるものの、その結果は十分に活用されているとはいえず、政策に患者の視点を反映させる方法は確立していない。

コモンズプロジェクトによる政策のエビデンス創出と患者参画
こうした背景のもと、私たちは2018年より「コモンズプロジェクト」（JST-RISTEX助成研究「医学・医療のためのICTを用いたエビデンス創出コモンズの形成と政策への応用」）を実施している。プロジェクトの目的は、希少疾患領域を中心とした医学・ヘルスケア領域の政策形成に資するエビデンスを創出すること、実践を通してエビデンス創出の効果的なステークホルダーの参画・協働の方法を探索することである。その成果として、患者さんとの共同執筆による国際論文が2023年11月に公開された。

コモンズプロジェクトでは、まず患者・患者家族、研究者、行政経験者が知識や経験を共有し、エビデンス創出に向けて継続的に熟議するための「エビデンス創出コモンズ」という場を構築した。最終的に、筋強直性ジストロフィー、骨格筋チャネル病、遺伝性血管性浮腫、MECP2重複症候群、ハンチントン病、脊髄小脳変性症、結節性硬化症、マルファン症候群、表皮水疱症、網膜色素変性症の10の希少疾患が対象となり、計43名が参加した。

研究テーマの優先順位設定
ステップ1：希少疾患患者が直面する困難の抽出
ステップ2：優先すべき研究テーマの「判断基準」の作成及び選定
ステップ3：「判断基準」の適用による研究テーマの優先順位設定

以上3ステップで計20回以上のワークショップを開催した。患者も共に研究を進める立場として参画し、議論の進め方や結果のまとめ方の検討、提言や論文執筆も共に行った。ステップ2では、今後の希少疾患研究を強化するために特に重要な「判断基準」として、「様々な側面のQOLに関するもの」「研究の成果によって患者本人の苦痛や負担を和らげ、自立に繋がるもの」の2つが選定された。さらにステップ3では、この2つの判断基準が当てはまる研究テーマとして、日常生活の支障、経済的な負担、就労や就学の悩み、不安や悲観、遺伝性疾患に特有の心理、通院の負担といった研究テーマが抽出された。

多岐にわたる希少疾患患者の困難は、既存の政策や研究だけでは十分に対応できていない。そのような困難に対し「コモンズ」では、ステークホルダーの視点を反映した優先順位設定を行った。つまり、ステークホルダー間の協働によるエビデンス創出という価値を生み出した。

また、予想以上に認識されたのが「コモンズ」という場そのものの価値であった。参加者が互いの視点や考え方を学び合い、自身の成長と信頼関係の醸成を実感した。そのことが議論の深化に大きく寄与し、「コモンズ」はより深い議論ができる場となった。

一方、「直面する困難を研究テーマとして捉える」ということが全員に十分理解されていないという課題も明らかになった。そこで、特定の研究テーマで具体的な研究内容を考えるための追加ステップを実施したり、「研究とは何か」を知るための研究者によるレクチャーを行ったりすることで徐々に理解が深まり、より深い議論に繋がった。このように、立場の異なるステークホルダーが協働する際には、理解や知識のギャップを埋めるための「翻訳」が重要である。

こうした研究の結果を基に、より患者の視点を反映した研究や政策を実現するために特に重要なことを次の３項目にまとめ、提言として公表した。

提言1：より多面的な患者の負担に焦点を当てた希少疾患研究の推進（患者やその家族のさまざまな負担の軽減や解決に繋がる研究を推進すること）
提言2：患者参画型の研究や活動の推進（患者参画型の研究の企画立案に関する助成など）
提言3：患者参画のあり方を考える研究の推進（患者参画の効果に関する評価方法を明らかにする研究など）

パネリスト

大坪 恵太（一般社団法人 日本難病・疾病団体協議会 事務局長）
古結 敦士
中島 唯善
西村 由希子
横田 正明

モデレーター

栗田 駿一郎（日本医療政策機構 シニアマネージャー）

セッションサマリー
パネルディスカッションでは、これからの難病対策として「診断ラグ」の解消を目指すことの重要性と、政策形成過程における患者・市民の参画に基づくアプローチの重要性が示唆された。診断ラグや早期診断の視点では、指定難病のあり方、社会への情報発信、早期診断を促進するための体制整備などについて議論が交わされた。特に自身の症状が難病かもしれないと「気づき」に至るまでの時間をいかに短くするかについては、現状の体制では不十分であり、今後の克服すべき課題であることが指摘された。また、難病政策の形成過程への患者・市民参画の視点では、ニーズに基づいた研究推進、地域協議会の設置とその機能強化などに関する議論が行われた。難病・希少疾患領域では、これまでも様々なレベルで患者参画が行われてきた。その取り組みの効果や意義を関係者が良く理解し、目的を見失うことなく推進することの重要性が指摘された。さらには、治療のみならず生活面にも注目した議論を展開することも期待される。

テーマ1：患者をいかに早く、医療やその他支援の枠組みにつなげるか（診断ラグの解消、早期対応の促進）

大坪 恵太

- ✓ 難病はその希少疾患としての特性から、知識や情報の不足、症状の多様性、高度専門的な検査の必要性などにより、いわゆる「診断ラグ」が生じている。特に地方では専門医が少なく、診断が遅れることで重症化するケースも多く、医療提供体制の地域差が大きな問題となっている。
- ✓ 難病・疾病団体協議会（JPA: Japan Patients Association）では年に1回、国会請願を行い、未診断疾患を含む難病の原因究明、診断基準と治療体制の確立、医療の地域格差の解消などを求めている。また、春と秋に国の審議会へ要望書を提出し、提言活動も行ってきた。
- ✓ さらなる課題解決に向けて、指定難病の数を増加させることが重要である。現状、341疾患が指定難病に指定されているが、小児慢性特定疾病は2倍以上の788疾病もある。小児慢性特定疾患のうち、指定難病の要件を満たすものを速やかに指定し、指定難病として研究を進めていく必要がある。

西村 由希子

- ✓ 難病領域は患者の数が少ないことが公的支援に繋がりづらい要因となっている。しかし、数が少ないから難病なのであり、少数意見も含めて「当事者の意見」として重視し、その結果をしかるべき組織に繋げていくことで、支援にも繋げていく必要がある。
- ✓ 希少疾患は病態の個別性や治療の専門性が高く、蓄積された知見や診断技術などの広がりに課題がある。救急医や精神科医など別の機会に難病・希少疾患を持つ患者との接点を持つ可能性のある医療者に対しても幅広く情報提供し、すべての医療者が診療に必要な知見・技術を獲得できるトレーニングが必要である。
- ✓ 患者サイドが必要とする情報が適時・的確に提供されるよう、適切な情報提供のあり方について、行政府、企業、患者などの関連するステークホルダーが協力して、議論を進めることが必要である。行政から発信される情報は正確であるものの、普遍的であり個別のニーズに即した適時的な情報発信がなされにくい。一方で、企業や研究者はより個別具体的かつ最新の情報を持ち合わせており、現行の制度上の規制を再検討し、患者目線で必要な最新情報が手に入るようにすべきである。

中島 唯善

- ✓ 難病の早期診断は日本医療研究開発機構（AMED）でも重視しており、診断がつかずに困っている患者（未診断疾患患者）を体系的に診断するためのプロジェクト（未診断疾患イニシアチブ（IRUD: Initiative on Rare and Undiagnosed Diseases））を推進している。こうした研究も含め、疾患の初期段階にも適用可能な治療の選択肢も明らかになってきており、幅広い症状の初期段階から治療・介入ができる仕組みを整備することも重要といえる。

横田 正明

- ✓ 厚生労働省では、難病の方が早期に適切な医療機関に繋がれるよう、医療提供体制の整備を進めている。特に、初診から診断に至るまでの期間をできるだけ短縮するため、「難病診療連携拠点病院」や「難病診療分野別拠点病院」の整備が重要であり、これらと身近な医療機関で医療の提供と支援する「難病医療協力病院」等との連携を通じて、早期診断の促進と適時・適切な医療機関への連携促進を目指している。
- ✓ 医療的な支援以外にも、例えば、難病の患者さんやご家族等からの相談に応じて対応を行う「難病相談支援センター」による支援や、小児慢性特定疾病を持つ児童やその保護者が抱える「ちょっとした悩み」を相談できるような相談支援事業も実施しています。また今回の法改正を通じて、小児慢性特定疾病児童等自立支援事業における実態把握に向けた取り組みを進め、地域のニーズ把握や課題の分析等を通じ、早期に適切な支援を実施できる体制づくりを目指している。

古結 敦士

- ✓ 患者が直面する課題や困難は多岐にわたり、既存の医療提供体制やその他の支援制度では十分にカバーできていない。今後は、患者が実際に抱える困難やその困難に対する具体的な対処法といった、患者に寄り添った研究の発展が求められている。
- ✓ （上述の）IRUDなどの取り組みにより、難病や希少疾患の可能性が指摘されてから正確な診断に至るまでの流れは整備されつつある。しかし、依然として患者自身や医療従事者が「難病かもしれない」と認識するまでには時間がかかっており、この部分を改善することが診断ラグ解消に向けた重要なポイントである。

テーマ2：難病政策の決定・推進における患者参画の推進に向けて課題や展望（難病対策地域協議会など）

横田 正明

- ✓ 現行の取り組みとして、患者への支援体制に関する課題の共有と連携等を目的としている難病対策地域協議会（以下、協議会）の設置・推進を行っている。設置は各自治体の努力義務とされているが、2023年4月1日時点で、都道府県レベルでは約9割、中核市などでは約5割が設置完了となっている。地方自治体担当者のマンパワー不足や業務過多などが、設置状況に差を生み出しているとみられ、課題となっている。
- ✓ 難病患者のニーズは多岐にわたるため、そうしたニーズに適切に対応するためには、地域における関係者の認識の共有が重要である。協議会で行われる議論は、政策に結びつく内容もあり、大事な場であると認識しており、今回の改正法では、小児慢性特定疾病児童等への支援の体制の整備を図るため、小児慢性特定疾病の協議会設置を法定化し、連携の強化を図っている。まずは今回の法改正による影響等を注視したい。

大坪 恵太

- ✓ JPAでは、協議会患者参画促進に向けて2つの取り組みを行っている。

  ①協議会未設置自治体への働きかけ：

  厚生労働省と連携し、要望書や請願を通じて協議会未設置自治体の解消を進めると共に、地域難病連として活動している36の加盟団体を通じて未設置自治体に設置に向けた要望を行っている。

  ②協議会の機能強化：

  協議会設置済みの自治体であっても、会議が年に1回しか開催されないなど「機能不全」といえる状況も散見される。参加者が広範にわたるため日程調整が難航するなど実務的課題も生じているため、関係者を限定した担当者レベルでの議論の場の設置推進などを厚生労働省などと連携し、課題解決に向けて取り組んでいる。

西村 由希子

- ✓ 協議会設置促進の取り組みと同時に、協議会を持続的なものにするための取り組みが必要である。協議会を持続可能なものにするには、活動内容やその評価方法についての指針を策定し、それに基づいて評価基準やガイドラインが策定されることで、一定の質が担保され、かつ主体的で持続可能な協議会活動の展開が期待される。
- ✓ また、患者参画と市民参画を明確に区別する事も重要である。患者参画は専門家等の関係者内での議論における患者の立ち位置を指すが、市民参画では患者を含む関係者全体がチームとなって、さらに市民との対話を重ねる必要がある。この違いを認識し、適切に使い分けることで課題に対して的確な議論が可能となるのではないか。

古結 敦士

- ✓ いわゆる「患者・市民参画」は、その重要性が社会的に認識されつつあるが、具体的な参画方法やその目的は未だ不明瞭である。多くの関係者は「どうすればよいか」という段階にあり、効果的な参画方法について研究や議論を深めていくことが求められる。
- ✓ 実際、患者・市民参画には様々な方法があり、参画方法により得られる結果や意義は変わる。例えば、広範囲のアンケート調査は「浅いレベル」の参画だが、多くの人々の意見を集められるという利点がある。一方、大阪大学で進めている「コモンズプロジェクト」（医学・医療のためのICTを用いたエビデンス創出コモンズの形成と政策への応用を目的とした研究プロジェクト）のように継続的な議論を通じて行う取り組みは、「深いレベル」の参画となるが、期間も長く、参加できる人数は限られる。これらの異なるアプローチを適切に組み合わせることで、より包括的な患者・市民参画が実現できるのではないか。

Kunio Tsuji (Standing Director, Japan Patients Association (JPA))

To commemorate the May 23, 2014 enactment of the Act on Medical Care for Patients with Intractable Diseases, or the "Intractable Disease Act," the Japan Patients Association (JPA) registered May 23 as "Intractable Disease Day." This year marks the tenth anniversary of the passage of that Act.

Estimates show that as of March 31, 2022, 1,050,000 people in Japan were receiving subsidies for healthcare costs associated with what the Government defines as "designated intractable diseases." There were 105,000 people living with "specified pediatric chronic diseases." Based on these figures, we can deduct that one out of every 100 people in Japan lives with some form of intractable disease.

Advances in treatment and research mean that a growing number of people living with intractable diseases are able to lead the same lifestyles as people without such conditions. However, some people experience recurrence, relapse, or progression of their condition that leads to various challenges, such as to their ability to remain in the workforce. Furthermore, only 30% of people living with intractable diseases hold certificates of disability, and I am often told that people are hesitant to tell potential employers about their intractable disease when searching for employment.

In the Basic Policy on Intractable Diseases, the Minister of Health, Labour and Welfare states the need to establish an environment that allows people living with intractable diseases to be able to disclose their condition with peace of mind as well as to balance treatment and work, but the current reality is that it is difficult for people to do so. Other problems in this area include regional disparities in healthcare provision and consultation support systems; school enrollment for children who require continuous medical care; challenges in the transitional phases from childhood to adulthood; and a lack of involvement for people living with intractable diseases in disability pensions and welfare for people with disabilities.

Intractable diseases can impact any citizen, and Japan enacted the Intractable Disease Act to support people living with intractable diseases and their families in its capacity as a mature society. In the past, Health and Global Policy Institute (HGPI) has presented policy recommendations focusing on health policy and various issues facing society, so expectations are high now that they have launched a project for intractable diseases. It is my sincere hope that today's symposium will be very fruitful, and that we can generate better recommendations thanks to the involvement of a diversity of stakeholders, starting with people most affected by intractable diseases.

Yukiko Nishimura (President, NPO ASrid)

In October 1972, when the Outline of Intractable Disease Measures was formulated, the term “intractable diseases” referred to rare diseases, or diseases that affect few people and are difficult to cure. In the Act on Medical Care for Patients with Intractable Diseases (hereinafter, the Intractable Disease Act), which was formulated 42 years later, the term came to encompass three items: rare diseases, intractable diseases, and long-term chronic diseases. While that law had been a long time in the making for related parties when it was enacted in 2014, the term “intractable disease” had already become a well-recognized concept in society. This created a slight gap between the legal definition of the term and how it is perceived by people living with intractable diseases and their families.

From September 30, 2023, to November 8, 2023, ASrid conducted a questionnaire survey of people living with intractable diseases and their families to gauge attitudes toward the new intractable disease measures introduced by the December 10, 2022 revision of the Intractable Disease Act. Items ranked favorably by respondents included “Provisions for retroactive billing that goes back to the emergence of severe symptoms” and “The issuance of registration certificates.” Issues that were pointed out included “The review of eligible parties and certification” and “The expansion of subsidies.”

ASrid has also been making progress in collaboration between the field of intractable diseases and other fields, as well as with the general public.

In June 2023, we established a group called the “Association for a Society with Easy Access to Clinical Trials for Everyone.” With an emphasis on being inclusive for everyone in discussions and on conveying requests appropriately, that group aims to discuss whether accurate information regarding clinical trials is being provided in an easy-to-understand manner. The people who originally started it serve on councils, at intermediary organizations, or as researchers or physicians in the fields of oncology, rare diseases, and intractable diseases. Its observers include the Ministry of Health, Labour and Welfare and the National Institute of Public Health, and the Japan Pharmaceutical Manufacturers Association serves as its secretariat. Together with such members, we recently submitted a request to the Government on the creation of a clinical trial database that is easy to access and understand.

Efforts to raise awareness for intractable diseases include a movement to designate a day as Rare Disease Day (RDD) that was launched in 16 European countries in 2008. RDD is now observed on the last day of February every year. As the largest social awareness event in this field, the aim of RDD is to improve QOL for people living with rare or intractable diseases and for their family members. The first RDD observed in Japan took place in 2010. RDD2024 marked its fifteenth anniversary with events held in 75 countries.

In addition to patient advocacy and support associations in various fields of disease, or hospitals and other organizations on the provider side of healthcare, the wide range of official hosts of RDD2024 also included libraries, middle schools, high schools, and shopping malls. RDD is characterized by the involvement of a wide variety of participants who actively disseminate information out of a desire to inform as many people as possible about rare and intractable diseases and to think about them together.

The saying, “Nothing about us without us” also applies to the area of intractable diseases, so in the future, we will continue working together with patients to consider what information patients need, and convey shared messages with everyone. This area is broad, so it may be difficult to encompass every intractable disease. However, we must have people affected by intractable diseases join our team and think together with us. It will be important for us to unite our voices when communicating to the general public and to continue putting action into motion.

Masaaki Yokota (Deputy Director, Intractable Disease Control Division, Public Health Bureau, Ministry of Health, Labour and Welfare (MHLW))

Key points of the Revised Intractable Disease Act and Revised Child Welfare Act

The revised Intractable Disease Act and revised Child Welfare Act include the expansion of suitable medical care and support for daily life while undergoing care for people like those living with intractable diseases or with specified pediatric chronic diseases; and the establishment of stipulations for databases for designated intractable diseases and specified pediatric chronic diseases. From last year to this year, these revised laws are being implemented in stages.

These revisions of measures for intractable diseases and pediatric chronic diseases are based on the written opinion of an MHLW deliberation council that includes members of patient advocacy groups for designated intractable diseases and specified pediatric chronic diseases that was presented in July 2021.

As for the main revisions, first, starting in October 2023, the timing of initial availability of medical expense subsidies was changed from the time of application to the time of onset of severe symptoms, and it was made possible for people to receive retroactive payments for up to three months prior to the date of application. Furthermore, to strengthen community support systems, the laws outline collaboration among Intractable Disease Consultation and Support Centers and people involved in welfare and employment support, enshrine the establishment of Regional Councils on Measures for Specified Pediatric Chronic Diseases into law, and obligate Regional Councils for Intractable Diseases and Regional Councils for Specified Pediatric Chronic Diseases to endeavor to collaborate.

In addition, in April 2024, a program was established to issue certification to people living with designated intractable diseases to facilitate the use of welfare, employment, and other support services. It also became possible to provide pharmaceutical companies and other such parties with anonymized data from the databases for designated intractable diseases and specified pediatric chronic diseases that has been processed so individuals cannot be identified. For example, when used as part of R&D at pharmaceutical companies, such data might be used to organize and grasp epidemiological information on specific groups, to collect new data on individual patients, or to understand and analyze information for approaching patients. We hope such information will be useful in drug discovery.

Promoting healthcare DX and the future direction for intractable disease measures

Given the importance of promoting the effective use of health, medical, and long-term care information to contribute to better health for individuals, to streamline operations in real-world healthcare settings, and to provide more efficient and effective healthcare and other services, the “Schedule for the Promotion of Healthcare DX” was compiled in June 2023 to achieve digital transformation (DX) in healthcare. Moving forward, we would like to improve the environment surrounding people living with intractable diseases and specified pediatric chronic diseases by advancing initiatives for healthcare DX according to that schedule.

For example, among ongoing efforts to integrate My Number cards with health insurance cards, a system that links medical subsidy data called Public Medical Hub (PMH) is being established so My Number cards can be used as proof of eligibility for medical subsidies. This will eliminate the need for people living with intractable diseases or similar parties to carry paper certificates, thus reducing the risk of losing or forgetting their certificates and the need to make multiple trips to health facilities. For health institutions, this system will eliminate the need for the manual entry of beneficiary information while providing access to up-to-date information on medical subsidy eligibility, thus reducing operational expenses associated with medical subsidy eligibility confirmation.

Another ongoing initiative is “Medical Service Fee Revision DX,” which aims to minimize burdens on health institutions by maximizing the use of digital technology. Efforts are underway to develop a shared calculation module (using standard medical claims computers) and to create master data for medical subsidy information for national and local public benefits. Disseminating the “Medical Service Fee Revision DX” system to all health institutions will make it possible to calculate local public benefits as benefits-in-kind as well as determine high-cost medical care expenses across health institutions (with no need for reimbursements). It will also make it possible to digitize the paperwork used for tracking upper out-of-pocket cost limits for local and national public benefits. These advances will ease the burden on health institutions while benefiting people living with intractable diseases.

It will also be important to promote the development of effective treatments or new pharmaceuticals and medical devices related to intractable diseases. While providing adequate protection to the rights and interests of the patients themselves, we will enable researchers and pharmaceutical companies to analyze health data and other information in the form of big data and improve the environment so the results of innovation in medicine and healthcare can be returned to citizens and patients. Efforts to examine the secondary use of healthcare information and other information are also advancing. For example, it may be possible to use or provide anonymized data when it has been determined there is considerable public interest in doing so. After implementing ample security measures, another possibility may be to establish a visiting analysis environment that allows the centralized and safe use and analysis of public databases.

In conclusion

As I mentioned in my introduction, the revised Intractable Disease Act and revised Child Welfare Act were enacted in 2022. Owing in part to related parties' preparations and cooperation, the content of those revised laws was implemented smoothly in October 2023 and April 2024. However, I think there are still items that remain to be addressed. Today, I gave an overview of the situation surrounding efforts to examine initiatives for healthcare DX. While continuing to gather opinions from related parties, in the future, I would like for us to advance each initiative to benefit people living with intractable diseases, people living with specified pediatric chronic diseases, and those close to them.

Presentation 3: AMED Initiatives for Rare and Intractable Diseases

Tadayoshi Nakashima (Program Officer and Research Officer, Division of Strategic Planning and Evaluation, Department of Innovative Drug Discovery and Development, the Japan Agency for Medical Research and Development (AMED))

AMED's support system for intractable disease research

In accordance with the second phase Plan for Promotion of Medical Research and Development, the Japan Agency for Medical Research and Development (AMED) has established a system that supports six integrated projects arranged by processes and types and that span the field of medicine from Basic Studies to Applied Studies for seven major disease areas (cancer, lifestyle related diseases, mental and neurological disorders, aging and dementia, rare and intractable diseases, child health and development, and infectious diseases).

The Practical Research Project for Rare / Intractable Diseases (the "Intractable Disease Project") has assigned personnel to five of those integrated projects (Pharmaceuticals; Medical Devices and Healthcare; Regenerative Medicine and Cell and Gene Therapy; the Project for Genome and Health Related Data; and Basic Medical Research).

In addition to designated intractable diseases, the Intractable Disease Project targets rare and/or intractable diseases that meet four conditions: unknown mechanism of onset, no established treatment method, rarity, and requiring long-term medical treatment. Aiming to overcome diseases by elucidating underlying causes and pathophysiology and by developing innovative diagnostic, therapeutic, and preventive methods, the Project supports research on approximately 200 items each year.

Characteristics of research in the field of rare and intractable diseases

Because rare and intractable diseases affect small groups of people, they have low marketability and it is difficult to establish businesses in these areas. This can make it difficult to get the private sector to lend its cooperation. It is also difficult to accumulate the necessary number of cases for regulatory approval, and development is very challenging. Furthermore, the underlying causes of these conditions are unknown, which hinders efforts to identify development targets. There are also some conditions for which evaluation methods have yet to be established.

Additionally, there are many unmet medical needs for intractable diseases that lack effective conventional treatment options. Another characteristic of this area is that many attempts are being made to respond to these conditions with the latest scientific technologies, such as nucleic acid medicine, gene therapy, and regenerative medicine. Furthermore, the absence of alternative treatments means that benefit-risk assessments sometimes differ from other diseases. Many of the researchers supported by the Intractable Disease Project are in academia. There are 341 designated intractable diseases and over 8,000 intractable diseases.

In March 2022, AMED conducted an investigation of current conditions and trends in Japan, the U.S., Europe, and China that examined regulatory measures for rare and/or intractable diseases as well as the circumstances and environment surrounding companies engaging in R&D. To make the most of those findings in designing a new call for proposals, AMED began providing support to researchers engaged in initiatives for ultra-rare diseases, utilizing real world data (RWD) registries, and precision medicine. AMED is also promoting the utilization of intractable disease research data through the Intractable Disease Platform and the Initiative on Rare and Undiagnosed Diseases (IRUD).

Recent achievements for the Intractable Disease Project include regulatory approval for Viltolarsen, a nucleic acid medicine for Duchenne muscular dystrophy (DMD); and Rituximab for systemic scleroderma and intractable pemphigus. In addition, in 2024, regulatory approval has already been obtained for three drugs, including Sirolimus for refractory vascular tumors and intractable vascular anomalies.

AMED activities undertaken in accordance with international goals

As the number of people living with rare and intractable diseases is small, international collaboration is also important. AMED serves as a member of the International Rare Diseases Research Consortium (IRDiRC) and is working to establish methods of providing definitive diagnoses, new treatment methods, and evaluation methods. As a result of these activities, AMED was able to grasp international circumstances around efficient methods for information sharing (standardizing terminology, data sharing, etc.) for rare disease phenotypes. This information was utilized to launch IRUD. Furthermore, by incorporating results of IRDiRC studies, such as to improve diagnostic accuracy for undiagnosed diseases, we achieved a diagnosis rate of 44%.

Promoting the co-creation of society through actions like Patient and Public Involvement (PPI) in research is particularly important in the field of intractable diseases. AMED requires researchers to fully consult patients from the stages of designing studies and clinical trial protocols.

Shizuko Tsushima (Director, Cancer and Disease Control Division, Healthcare and Medical Services Department, Health and Medical Services Bureau, Kanagawa Prefectural Government)

Intractable disease measures in Kanagawa Prefecture

It has been reported that as of March 31, 2024, approximately 60,000 people in Kanagawa Prefecture had been diagnosed with and met certain criteria for designated intractable diseases. As of April 1, 2024, 341 diseases in 14 disease groups had been identified.

The support system for people living with intractable diseases in Kanagawa Prefecture is centered on the Kanagawa Intractable Disease Consultation and Support Center. It provides Kanagawa residents living with intractable diseases and other community members with consultation and support on daily life, opportunities for community exchange, and employment support. It is operated by National Hakone Hospital, which has a long track record in treating neurological intractable diseases. (It has been jointly operated under commission by the prefectural government and three designated cities since FY2017.) In FY2023, the Center provided 1,239 consultations that covered information related to all aspects of daily life while undergoing medical treatment; guidance for healthcare institutions and systems; and patient groups or peer support.

Consultations are also provided at public health centers in the region. Public health centers and municipalities work together to maintain connections throughout the region by sharing information and collaborating through the Regional Council for Intractable Disease Control; Hello Work offices, which provide employment consultation; and the Integrated Occupational Health Support Center, which provides support for balancing health and work.

Kanagawa Prefecture has established a healthcare provision system for intractable diseases that includes four university hospitals designated as coordinating base hospitals for intractable disease care. It has also defined eight medical regions in the prefecture which include a total of 29 hospitals designated as supporting hospitals for intractable disease care. Kanagawa Prefecture has also established a system for mutual collaboration that can be used to coordinate patient intake, including with other general hospitals and clinics; to provide training; and to provide medical information. For people who would like respite care, there are nine hospitals that provide it (excluding those that provide their own). Three new hospitals were designated as supporting hospitals for intractable disease care in FY2023, which has helped to alleviate the uneven distribution of patients in the region. Kanagawa Prefecture has also established a consultation support system centered on these facilities, public health centers, and municipalities.

Future issues identified at the Council for Intractable Disease Control

The Council for Intractable Disease Control has identified a total of six issues, which include: establishing a seamless care provision system for people living with an intractable disease since childhood; determining how the Intractable Disease Information Collaboration Center and base hospitals can best share information; and implementing disaster measures for people living with intractable diseases.

Among these issues, discussions on “establishing a seamless care provision system for people living with an intractable disease since childhood” are advancing alongside ongoing discussions on the establishment of a system for healthcare during transitional phases. A number of needs and issues were identified at the first Council meeting, which was held in FY2023. These included: the need to support people living with intractable diseases when entering school or employment, during pregnancy or childbirth, and at other such life events; insufficient cooperation for patients among health institutions; the lack recognition toward the existence of or issues facing transitional phases for conditions that are specified pediatric chronic diseases but are not designated intractable diseases; and the need to reinforce collaboration among relevant Government departments in pediatric chronic diseases and intractable diseases.

Kanagawa Prefecture initiatives for pediatric chronic diseases include the Project to Support Independence for Children Living with Chronic Diseases and the Project for Establishing a Healthcare Support System for Transitional Phases. The former aims to leverage social resources in communities to provide support that is tailored to users’ environments and other characteristics. The latter project aims to develop a system providing medical support during transitional phases, such as by establishing support systems that involve collaboration among healthcare professionals responsible for each period of life, from childhood to adulthood; or by providing support for autonomy and independence through activities like helping users improve their understanding toward their own conditions.

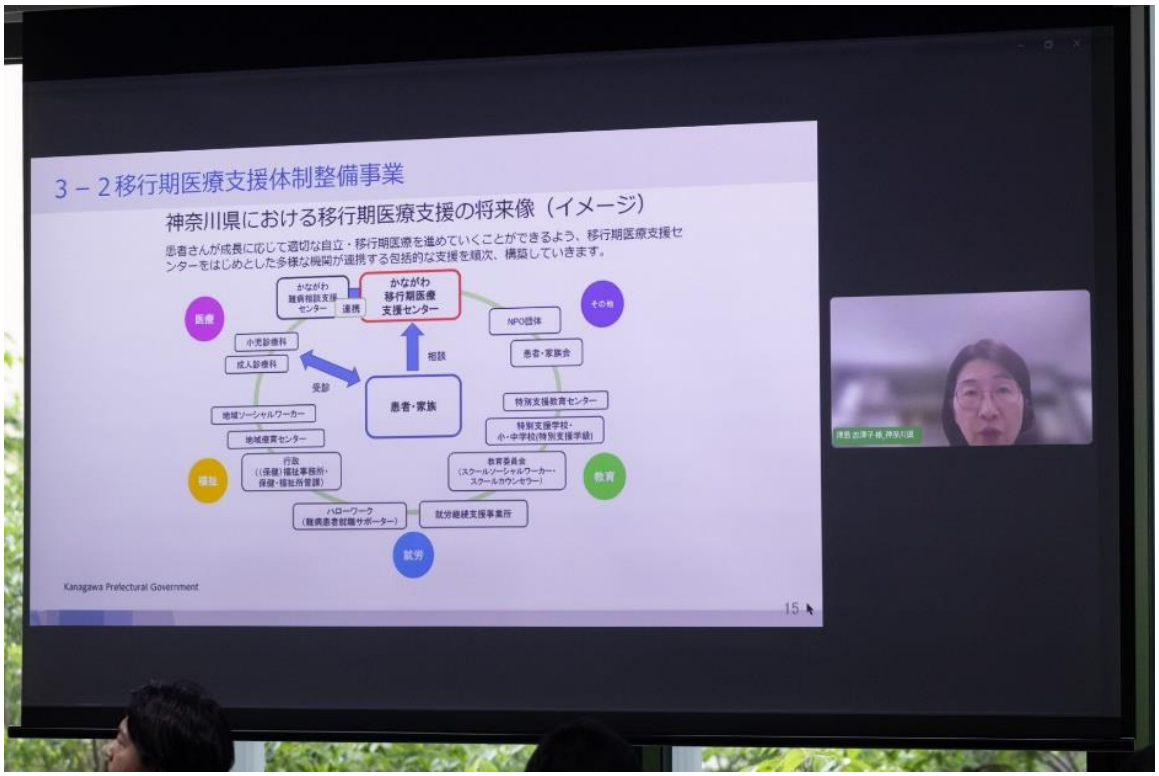
When people with specified pediatric chronic diseases enter adolescence and adulthood, they should be transferred from pediatric departments to adult departments so they can receive suitable care. I believe that we need to create an integrated system that provides support from childhood to adulthood, so that appropriate health care and support for independence can be provided at each life stage.

Building a seamless healthcare provision system

The Kanagawa Transitional Healthcare Support Center was established by the National Hakone Hospital under commission from the Project for Establishing a Healthcare Support System for Transitional Phases. Through its main activities, which include providing consultation support by transitional care support coordinators (who provide advice for independence, information on various systems, etc.) and establishing and maintaining a transitional care system that coordinates collaboration among pediatric and adult departments (such as by collecting and publishing information on adult health institutions in each region or medical field), the Kanagawa Transitional Healthcare Support Center is now building up a track record of successful consultations.

Lectures on healthcare are also being hosted once a year both in person and online for health and administrative institutions in the prefecture. These events provide opportunities to gather opinions from various parties.

As for our future vision for healthcare support in transitional phases in Kanagawa Prefecture, we plan to continue building an integrated support system with collaboration among various institutions including the Kanagawa Transitional Healthcare Support Center so that community members can become more independent and continuously receive appropriate transitional healthcare as they grow.



Atsushi Kogetsu (Assistant Professor, Department of Biomedical Ethics and Public Policy, Graduate School of Medicine, Osaka University)

Background of Patient and Public Involvement (PPI) in healthcare, research, and policy

As advances are made in science and technology and healthcare becomes more sophisticated, there has been growing recognition toward the gap between healthcare, research, and policy, which are led by experts, and the challenges and needs of healthcare beneficiaries. Given this context, over the past 20 years, the field of healthcare has seen an expansion of Patient and Public Involvement (PPI) in real-world healthcare settings and in medical research. This occurred partly because of growing citizen movement and patient-led activities, as well as the promotion of PPI as a matter of national policy. In 2017, INVOLVE, which was established by the National Institute for Health and Care Research in the UK to support public involvement in the fields of healthcare and welfare, defined PPI as, "Research being carried out 'with' or 'by' members of the public rather than 'to', 'about,' or 'for' them."

In Japan, the movement for PPI began in the late 2000s, mainly in the field of oncology. More progress for PPI in research was made in 2014, with the start of RUDY JAPAN, which had patient involvement; the launch of J-RARE, which is a patient-operated rare and intractable disease patient registry platform; and efforts for PPI in clinical research on IPS cells (Muto et al.). Later, AMED and other national organizations also began taking part in promoting PPI.

In recent years, there has been growing recognition toward the importance of having evidence in some form when formulating and implementing policies. Simultaneously, more people are starting to recognize the importance of involving the people most affected by policy in those processes. As one concrete example, in the health sector, focus has been placed on involvement from patients when setting research priorities. However, despite the presence of existing examples of involvement, the results of those efforts have not been fully utilized, and methods of reflecting the perspectives of patients in policies have yet to be established.

Establishing evidence and patient involvement in policy through the Commons Project

Against this backdrop, starting in 2018, our team has been advancing an initiative called the Commons Project. It is funded by the Japan Science and Technology Agency Research Institute of Science and Technology for Society (JST-RISTEX) under the title, "Constructing a commons utilizing ICT to generate evidence for medical policy." The Commons Project aims to generate evidence that contributes to policy formulation in the fields of medicine and healthcare, particularly for rare diseases; and to examine effective methods of involvement from and collaboration with stakeholders in generating evidence through practical efforts. One result of the project was an international paper that was co-authored with patients and was published in November 2023.

The Commons Project began an initiative for patients and their families, researchers, and former policymakers to share their knowledge and experience and to hold continuous discussions on generating evidence by establishing a forum called the "Evidence Generating Commons." The project ultimately included 43 participants and covered ten rare disease areas: myotonic dystrophy, skeletal muscle channelopathies (non-dystrophic myotonias), hereditary angioedema, MECP2 duplication syndrome, Huntington's disease, spinocerebellar degeneration, tuberous sclerosis, Marfan's syndrome, epidermolysis bullosa, and retinitis pigmentosa.

Priority research topics were determined using a three step process:
Step 1: Clarification of difficulties faced by patients with rare diseases
Step 2: Development and selection of criteria for priority setting
Step 3: Priority setting through the application of the criteria

Over the course of these three steps, we held a total of over twenty workshops. In addition to being involved in advancing the study, the patients who participated also took part in examining how to advance discussions, compiling findings, and co-authoring the recommendations and report. Two of the criteria developed in Step 2 were used to identify research topics that will be especially important for strengthening future rare disease research: those related to various quality of life (QOL) aspects, and those related to expected findings that are likely to alleviate patients' pain and burden and lead them to gain independence. Furthermore, in Step 3, we identified research topics for which those two criteria apply: impediments to daily life, financial burden, concerns about working and schooling, anxiety, pessimism, mental state specific to genetic diseases, and burden of hospital visits

The challenges faced by people living with rare diseases come in many forms and we have not been able to adequately address them through existing policies and research alone. The Commons set priorities that reflect the viewpoints of stakeholders to address those challenges. In other words, we created value in the form of evidence generated through collaboration among stakeholders.

Another item that received more recognition than we anticipated was the value of the Commons itself. Participants learned from each other's perspectives and ideas, and they were able to feel their own growth and that they were fostering trusting relationships. This greatly contributed to deepening discussions and helped make the Commons a forum where deeper discussions could be held.

On the other hand, it became clear that not all participants fully understood the concept of perceiving the difficulties directly facing people with rare diseases as research topics. To address this, we took additional steps to consider the specific content of studies for specific research topics, and through lectures by researchers on what research is, we were able to gradually improve understanding, which led to deeper discussions. As these experiences taught us, translation is important for bridging gaps in understanding and knowledge when engaging in collaboration with stakeholders from different positions.

Based on our research, we compiled and presented the following three recommendations on items that are particularly important for conducting research and generating policies that better reflect patients' perspectives.

Recommendation 1: Promote rare disease research that focuses on patient burden in a more multifaceted manner. (In other words, promote research that aims to lighten the various burdens on patients and their families or that will lead to solutions.)
Recommendation 2: Promote research and activities that include patient involvement. (For example, by providing funding for proposals for studies with patient involvement.)
Recommendation 3: Promote research that considers the ideal structure of patient involvement. (For example, research that identifies methods of evaluating the effects of patient involvement.)

Panelist

Keita Otubo (Secretary General, Japan Patients Association (JPA))
Atsushi Kogetsu
Tadayoshi Nakashima
Yukiko Nishimura
Masaaki Yokota

Moderator

Shunichiro Kurita (Senior Manager, HGPI)

Session summary

The panel discussion indicated that eliminating diagnosis lag will be an important part of future intractable disease measures and recognized the importance of adopting an approach based on involving people living with intractable diseases and citizens in the policy formulation process. As for addressing diagnosis lag and providing early diagnosis, topics discussed included the nature of designated intractable diseases, methods of transmitting information to society, and the establishment of a system to promote early diagnosis. In particular, one important future issue to address is that existing systems are inadequate for shortening the time required for people to recognize that their symptoms may be caused by an intractable disease. From the perspective of involving people living with intractable diseases and citizens in the intractable disease policy formulation process, items discussed included the promotion of research that is based on the needs of those most affected and establishing and strengthening the functions of regional councils. In the past, the area of intractable and rare diseases has had involvement for those most affected at various levels. During the discussion, it was pointed out that it is important for related parties to fully understand the effectiveness and significance of involvement and to promote involvement without losing sight of its purpose. Finally, participants shared their expectations for future discussions to not only focus on treatment, but to also cover aspects related to daily life.

Theme 1: Providing people with timely connections to healthcare and other support frameworks (eliminating diagnosis lag and promoting early response)

Keita Otsubo

- ✓ As intractable diseases are rare diseases, what is referred to as “diagnosis lag” occurs due to aspects that are common to rare diseases. These include insufficient knowledge and information, the diversity of symptoms, and the need for highly-specialized testing. In particular, many patients develop severe conditions due to diagnostic delays that occur because of shortages of specialist physicians outside of major metropolitan areas, and regional disparities in healthcare provision systems have become a major problem.
- ✓ Once a year, the Japan Patients Association (JPA) submits a request to the Diet on identifying the underlying causes of intractable diseases, including undiagnosed diseases; establishing diagnostic criteria and treatment systems; and eliminating disparities in healthcare among regions. JPA also submits written requests to the national council in spring and fall and engages in advocacy activities.
- ✓ Increasing the number of designated intractable diseases will be an important step in furthering efforts to address issues. Currently, 341 diseases are designated intractable diseases, but there are 788 specified pediatric chronic diseases—more than twice as many. Specified pediatric chronic diseases that meet the criteria to be designated intractable diseases must be designated quickly and research on them as designated intractable diseases must be advanced.

Yukiko Nishimura

- ✓ In the area of intractable diseases, the fact that there are few patients is one factor that hinders efforts to connect them with public support. However, these conditions are intractable because patient numbers are low, and we must emphasize the opinions of people living with intractable diseases, even when their opinions represent only a few people. Our findings must then be used to connect affected parties to the organizations and support they need.
- ✓ The pathophysiologies of rare diseases are highly individualized, they require highly-specialized treatment, and there are challenges in disseminating accumulated knowledge, diagnostic techniques, or other relevant information. Information must be provided to a wide range of health professionals who may come into contact with patients with intractable or rare diseases on other occasions, such as emergency physicians and psychiatrists, and training must be provided to equip all health professionals with the knowledge and skills necessary for providing treatment.
- ✓ Ensuring that those on the patient side are provided with the accurate information they need and at the right times will require advancing discussions on how to best provide information with cooperation from relevant stakeholders such as the government, companies, and patients. While information provided by the government is accurate, it is universal in nature, and it is difficult for the government to provide information that is tailored to the needs of individuals in a timely manner. However, industry and researchers have more individualized, specific, and up-to-date information, so existing regulations within the system should be reexamined to ensure the availability of up-to-date information that is necessary from the viewpoints of patients.

Tadayoshi Nakashima

- ✓ Early diagnosis for intractable diseases is also a priority item at the Japan Agency for Medical Research and Development (AMED). AMED is currently advancing a project called the Initiative on Rare and Undiagnosed Diseases (IRUD), which aims to systematically diagnose people who are struggling with undiagnosed diseases. Various initiatives including that study are gradually discovering treatment options that can be implemented during early stages of diseases, so it is also safe to say that it will be important to establish a framework for providing treatment and intervention starting in the early stages for a wide range of symptoms.

Masaaki Yokota

- ✓ The Ministry of Health, Labour and Welfare (MHLW) is advancing efforts to establish a healthcare provision system that will connect people living with intractable diseases to appropriate healthcare institutions at early stages. Establishing base hospitals for intractable diseases and that specialize in specific fields of intractable diseases will be a particularly important step in minimizing the time it takes for people to receive diagnoses from their initial medical examinations. Through collaboration with facilities such as cooperating hospitals for intractable disease treatment and other health facilities that are close to those base hospitals and that support healthcare provision, the MHLW aims to promote early diagnosis and timely and appropriate collaboration with healthcare institutions.
- ✓ The MHLW is also engaged in a number of other initiatives outside of support related to medical services. For example, there is support being provided to people living with intractable diseases and their families by Intractable Disease Consultation Support Centers. Consultation support services are also being provided to help address the smaller troubles experienced by children with specified pediatric chronic diseases and their guardians. Furthermore, through the recent legal revisions, the MHLW is also advancing efforts to grasp real-world circumstances in the Project to Support Independence for Children Living with Specified Pediatric Chronic Diseases with the aim of establishing a system that can provide suitable support at early stages by understanding what needs are present in communities and by analyzing the issues that communities face.

Atsushi Kogetsu

- ✓ The issues and challenges faced by people living with intractable diseases are diverse, and are not fully covered by the healthcare provision system and other support systems in their current forms. In the future, we must expand research that is closer to patients and that aims to identify the real issues facing patients and concrete methods of addressing them.
- ✓ Progress is currently being made on establishing processes from the identification of potential intractable or rare diseases to the provision of accurate diagnoses through previously-mentioned initiatives such as IRUD. However, it still takes time for those most affected and healthcare professionals to recognize that the condition of the affected party is an intractable disease. Making improvements during that part of the process will be a key point for eliminating diagnosis lag.

Theme 2: Challenges and future prospects for promoting involvement from people living with intractable diseases in formulating and advancing intractable disease policy (such as through Regional Councils for Intractable Disease Control)

Masaaki Yokota

- ✓ One current initiative is the establishment and promotion of Regional Councils for Intractable Disease Control (hereinafter, “councils”). Each local government has been obligated to endeavor to establish these councils, which exist so that patients’ issues related to support systems can be shared and so collaboration can take place. As of April 1, 2023, councils have been established by approximately 90% of prefectural governments and around 50% of core cities and other such municipalities. It is likely that issues like insufficient manpower and heavy workloads for officials at local governments who can lead such efforts have resulted in gaps in the implementation status of these councils, and these issues must also be addressed.
- ✓ The needs of people living with intractable diseases are diverse, so having a shared recognition among relevant parties in communities will be important for mounting appropriate responses to meet those needs. The discussions held at councils include content that is linked to policy, and they provide key opportunities for building that recognition. To improve the system for supporting children living with specified pediatric chronic diseases, the recently-enacted revised law makes it a legal requirement to establish pediatric chronic disease councils, which will strengthen collaboration. I would like for us to begin by paying close attention to the impact of the revised law.

Keita Otsubo

- ✓ JPA has been advancing two initiatives to promote the involvement of those most affected in councils:

  1) Approaching local governments that have yet to establish councils

  JPA is working together with the MHLW to ensure that no municipality goes without establishing a council by submitting written requests and petitions. JPA is also submitting such requests to municipalities without councils through its Regional Federation for Intractable Diseases, which consists of 36 member organizations.

  2) Reinforcing council functions

  Among municipalities where councils have already been established, there are some cases in which councils are not functioning properly due to practical issues. For example, some councils only meet once per year because they include a wide range of participants and face scheduling difficulties. JPA is working together with the MHLW and other partners to address these issues, such as by establishing forums for discussion at the council leadership level with attendance limited to related parties.

Yukiko Nishimura

- ✓ Alongside initiatives to encourage the establishment of councils, it will also be necessary to introduce initiatives that aim to ensure councils are sustainable. To help make councils sustainable, after creating guidance on council activity content and evaluating council activities, evaluation criteria and guidelines should be established based on that guidance. Doing so is likely to help ensure that quality meets certain standards as well as foster proactive and sustainable council activities.
- ✓ It will also be important to clearly distinguish between involvement for people living with intractable diseases and involvement for citizens. Involvement for people living with intractable diseases refers to the position of affected parties in discussions with experts and other related parties. Meanwhile, citizen involvement requires forming teams that encompass all related parties, including those living with intractable diseases, and holding repeated dialogues with citizens. Being aware of this difference and making appropriate use of the two terms may allow for more accurate discussions on issues.

Atsushi Kogetsu

- ✓ There is growing recognition from society of the importance of involvement from people living with intractable diseases and citizens, but specific methods of involvement and its purpose are still unclear. Many stakeholders are still at a stage where they are unsure about how to incorporate involvement, so we must conduct more research on effective involvement methods and further deepen discussions.
- ✓ In fact, there are a variety of methods of involving people living with intractable diseases and citizens, and the results of involvement and its significance varies by method. For example, a questionnaire survey that targets broad portions of the public may be a shallow form of involvement, but such surveys offer the benefit of collecting the opinions of many people. On the other hand, there are also forms of deeper involvement that occur through continuous discussions, such as the “Commons Project,” a research project at Osaka University that aims to construct a commons utilizing ICT to generate evidence for health policy. However, such initiatives take great amounts of time and the number of people that can participate is limited. By appropriately combining these different approaches, it may be possible to achieve more comprehensive forms of involvement for people living with intractable diseases and citizens.

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