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Book review

Weronika Wojturska*

Ready for risk sharing? Performance-based agreements as a catalyst of innovative pharmacotherapy**

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Abstract

Since health authorities and pharmaceutical companies are risk-averse in the reimbursement process, risk-sharing agreements (RSAs) can be seen as a happy medium for ensuring that the inherent financial and clinical risks of implementing a new innovative treatment are minimized. Although a properly applied RSA lies in the interest of patients, payers and manufacturers, the initial analysis shows that implementation of performance-based RSAs (PBRSAs), tied to measurable health outcomes, faces numerous obstacles in Poland. The article seeks to propose solutions, taking into account the characteristics of PBRSAs in Italy and the United Kingdom. The analysis is in line with the current trend of gradual remodelling of the Polish system towards value-based healthcare. The methods employed include a broad conceptual and exploratory analysis of Polish and foreign literature and legal acts, data acquired from national health data repositories, materials published by pharmaceutical consortia, financial institutions and public authorities engaged in managing the medicine market in Europe.

Keywords
risk-sharing agreement, RSA, performance-based RSA, PBRSA, reimbursement, pharmacotherapy

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Introduction

Health systems in industrialised countries constantly balance between controlling expenditure, fostering innovation, and providing access to new diagnostic and therapeutic options. This challenge is not made any easier by the high incidence of medicine shortages in most European countries.[1] The problem is particularly significant in the case of oncology patients, where it is often difficult to assess the value of innovative drug therapies because of the uncertainty about their clinical benefit and cost effectiveness. As a result, payers may be unwilling to pay the pharmaceutical companies' list prices.

In countries where the public sector plays a dominant role in the provision/reimbursement of medicines, policymakers may regulate drug prices to address this issue, while providing some form of monetary value measurement. It may be based on price referral, performance of health technology assessments, control of profit margins on medical and pharmaceutical products or co-payments for pharmaceutical dispensing.[2] At the same time, new methods are being explored, such as performance-based pricing. It is implemented through risk-sharing agreements (RSAs) between payers and pharmaceutical companies, whereby the risks linked to the clinical and economic performance are shared and remuneration for a drug is dependent on its real-world value.[3]

The potential of RSAs has been recognised in Poland. It is a way of relieving the state budget, as in most cases manufacturers return the difference between the official selling price of patient treatment and the so-called effective price[4] directly to the National Health Fund (NHF) budget. Although properly applied RSA is in the interest of patients, payers and manufacturers the initial analysis shows that the implementation of performance-based RSAs (PBRSA), which are focused on delivering improved health economic value, transcending fixed-cost-per-unit and rebating practices, faces numerous obstacles in Poland. The article seeks to propose solutions, taking into account the characteristics of PBRSAs practice in Italy and the United Kingdom. The analysis is in line with the current trend of gradual remodelling of the Polish system towards value-based healthcare.

The methods employed include a broad conceptual and exploratory analysis of Polish and foreign literature and legal acts, data acquired from national health data repositories, as well as external online desk research of the materials published by pharmaceutical consortia, financial institutions and public authorities engaged in managing the medicine market in Europe.

1. See: Pharmaceutical Group of the European Union, *Medicine Shortages Survey 2022*, https://www.pgeu.eu/wp-content/uploads/2023/01/Medicine-Shortages-PGEU-Survey-2022-Results-1.pdf, (access 01.06.2023). The observed gap in needed information, tools and legal solutions for providing adequate support to patients in case of a shortage increases the political pressure on the EU to come up with a bloc-wide response. The European Commission will draw up guidance for national stockpiles in 2024 and establish a Critical Medicines Alliance, bringing governments, industry and civil society together. In addition, the European Medicines Agency is developing a list of critical medicines by the end of 2023 to spot shortages as they occur and to help identify and coordinate available alternatives, see: *Medicine shortages and availability issues*,https://www.ema.europa.eu/en/human-regulatory/post-authorisation/availability-medicines, (access 01.06.2023). In Poland, however, a national register of shortages for human medicine has not been established as yet, although the Polish Supreme Pharmaceutical Chamber declares that it provides such data to the Ministry of Health, just as wholesalers and pharmacies report their stocks and purchases.

2. F.R. Gonçalves, S. Santos, C. Silva, et al., *Risk-sharing agreements, present and future*, "Ecancermedicalscience", 2018,

Terminology and typology

There are several factors affecting the healthcare budget, such as the patient population, the duration of treatment, dosage and the actual clinical efficacy of a drug. Adding a new medicine to the list of price-regulated and publicly reimbursed medicines is always associated with uncertainty. Clinical trials may not always correctly predict the real effectiveness of a drug.[5] In particular, this is the case for new, innovative and expensive drugs targeted at small populations. In those cases, clinical studies are small and provide inconclusive evidence. This leads to outcome uncertainty as regards a patient's response to treatment and the resulting health outcomes.[6]

From the payer's point of view, this uncertainty is increased by the "experimental" character of every newly launched drug. There is a risk of unequal access to information on the results of the implementation of a new drug's between the pharmaceutical company and the cost-payers, especially regarding the number of prescriptions made out by doctors and valuation of the beneficiaries. As a result, pharmaceutical companies have trouble with controlling the expected profit margin. Given their limited budget, the payers might want to avoid the risk of investing in a new technology, and insist on investing only in drugs that have been proven to be most effective.[7]

Payers and pharmaceutical companies have different priorities when it comes to pricing new therapies. Payers seek the optimal value for money across a number of different available therapies, while pharmaceutical companies look for a refund of their investment in research and development of a new drug. Nonetheless, both sides can profit from an objective assessment of the related costs and benefits.

Over the past two decades, due to the pressure on payers (controlling costs and ensuring cost-effectiveness) and manufacturers (ensuring access to formulations), a new paradigm for risk sharing has been developed, known under the generic name of RSA. Although risk-sharing mechanisms, rebate and payment rules may vary, the basis is always the same – linking payment to the actual value of a medical technology. Not surprisingly they are predominantly arranged within oncology (52%), since these drugs typically rely on RSAs to get reimbursement.[8]

There is a number of studies that comprehensively review existing and historical RSAs proposing several taxonomies detailing their subtleties.[9] Therefore, there is still confusion over terminology. Towse and Garrison were the first to suggest an initial taxonomy as "agreements between a payer

Vol. 12, p. 2, DOI: 10.3332/ecancer.2018.823.

3. C. Buch, J. Schildmann, J. Zerth, *Risk-sharing schemes to finance expensive pharmaceuticals: Interdisciplinary analyses*, in: *Defining the Value of Medical Interventions: Normative and Empirical Challenges*, eds. J. Schildmann, C. Buch, J. Zerth, W. Kohlhammer GmbH 2021, https://www.ncbi.nlm.nih.gov/books/NBK585090/#ch07sec1_2, (access 05.06.2023).

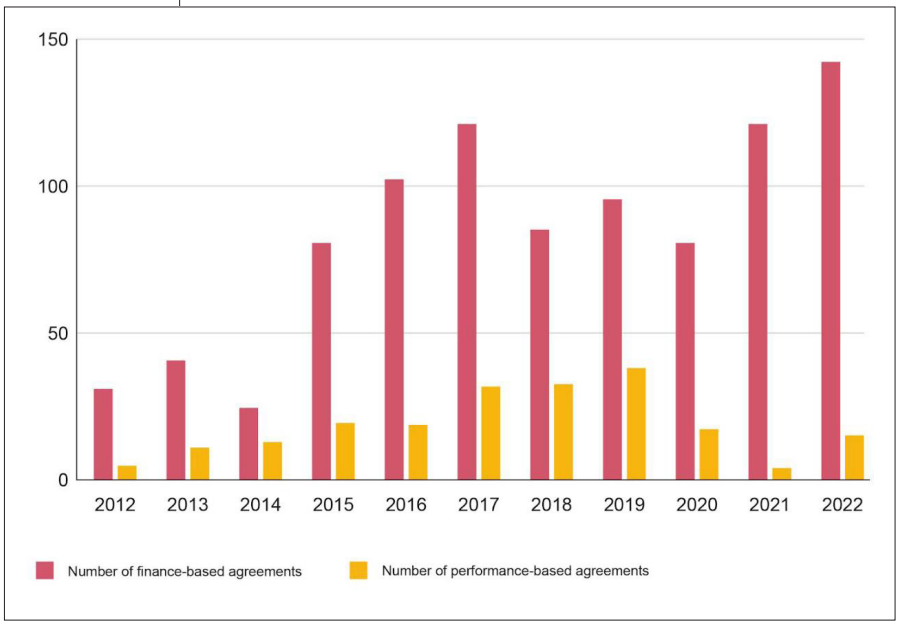
4. The price of a drug obtained as a result of a reduction in the official selling price by a risk-sharing instrument.

5. L. Garrison, A. Towse, A. Briggs, et al., *Performance-based risk-sharing arrangements-good practices for design, implementation, and evaluation. Report of the ISPOR good practices for performance-based risk-sharing arrangements task force*, "Value in Health", 2013, Vol. 16 (5), p. 704, DOI: 10.1016/j.jval.2013.04.011.

6. J.J. Carlson, S.D. Sullivan, L.P. Garrison, et al., *Linking payment to health outcomes: a taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers*, "Health Policy", 2010, Vol. 96 (3), p. 188, DOI: 10.1016/j.healthpol.2010.02.005.

7. A. Towse, L. Garrison, *Can't get no satisfaction? Will pay for performance help? Toward an economic framework for understanding performance-based risk sharing agreements for innovative*

and a pharmaceutical company where the price level and/or revenue received is related to the future performance of the product in either a research or real-world environment."[10] In contrast, I consider the one proposed by Adamski et al. to be a more coherent definition that follows the principles of a logical division.[11] The authors assume that there is only one basis of the division: every subject belongs to one group only, where there may be sub-categories of equal rank, and every example from a superior group, i.e. risk-sharing schemes, has to fall into either category, i.e. either finance-based or performance-based.[12] Therefore, RSAs should be considered as "agreements concluded by payers and pharmaceutical companies to diminish the impact on payers' budgets of new and existing schemes brought about by uncertainty and/or the need to work within finite budgets."[13] Pharmaceutical companies grant some kind of warranty for the value of a medical drug. The agreed condition of this "risk" varies by situation, and can include pharmaceutical expenditure higher than agreed thresholds or lower than expected health gain from a new product. In practice, such a contract may under certain conditions reduce the net financial cost to the payer. This differs from the traditional approach, in which health authorities assumed almost all the risk.



Source: A. Watt, *Risk-sharing...*, op. cit.

According to GlobalData's risk-sharing database, over 1,000 RSAs were made out in the last decade across 28 countries involving roughly 100 companies.[14]

Figure 1. Global distribution of RSAs by year and type

An overwhelming 79% of known RSAs are finance-based, ranging in complexity from simple discount schemes to more complex risk-based financial arrangements. This type does not usually take a patient outcome into account, but rather concentrates on setting caps or limits on the amount spent per product or patient. While performance-based RSAs (PBRSA) that establish a threshold indicator that must be achieved before ad-

medical products, "Pharmacoeconomics", 2010, Vol. 28 (2), pp. 93–94, DOI: 10.2165/11314080-000000000-00000.

8. A. Watt, *Risk-sharing agreements are growing at a rate of 24%*, 2023,https://www.pharmaceutical-technology.com/pricing-and-market-access/risk-sharing-agreements/?cf-view, (access 03.08.2023).

9. Cf. P. Kanavos, A. Ferrario, G. Tafuri, et al., *Managing risk and uncertainty in health technology introduction: the role of managed entry agreements*, "Global Policy", 2017, Vol. 8 (2), pp. 84–92, DOI: 10.1111/1758-5899.12386; T. Stafinski, Ch.J. McCabe, D. Menon, *Funding the unfundable - mechanisms for managing uncertainty in decisions on the introduction of new and innovative technologies into healthcare systems*, "Pharmacoeconomics", 2010, Vol. 28 (2), pp. 113–142, DOI: 10.2165/11530820-000000000-00000; J.J. Carlson, S.D. Sullivan, L.P. Garrison, et al., *Linking payment...*, op. cit., pp. 179–190; C. McCabe, T. Stafinski, R. Edlin, et al., *Access with evidence development schemes - a framework for description and evaluation*, "Pharmacoeconomics", 2010, Vol. 28 (2), pp. 143–152, DOI: 10.2165/11530850-000000000-00000; C. McCabe, L. Bergmann, N. Bosanquet, et al., *Biotherapy Development Association, Market and patient access to new oncology products in Europe: a current, multidisciplinary perspective*, "Annals of Oncology", 2009, Vol. 20 (3), pp. 403–412, DOI: 10.1093/annonc/mdn603.

ditional payment is made or a rebate is offered initially grew in popularity, ease of implementation allowed finance-based models to dominate. In the following discussion, the focus will be on PBRSAs, also named outcome or value-based contracts, which should be perceived as complex RSAs, as opposed to finance-based (simple) RSAs.

Characteristics of PBRSAs

PBRSAs as an innovative contractual arrangements represent a viable coverage and reimbursement mechanism for a wide range of medical products.[15] They are often framed and characterised as applicable to therapies with measurable outcomes, which can be both established drugs or new entrants seeking market expansion.[16] Their principal objective is to solve access problems faced by countries and manufacturers through strategic partnerships. Payers benefit through cost containment, ensuring better value for money and easier access, potentially leading to better health outcomes for the covered population. Manufacturers secure market access at or near launch and achieve more efficient global pricing strategies. The long-term success of these arrangements depends on the ability of the parties to develop mutually beneficial arrangements that entail a minimal administrative burden in their development and implementation. In addition, this type of instrument is a source of data that can be used for academic work and clinical activities, including the running of even complex and niche drug programmes primarily by large research-oriented hospitals.

Performance can be understood by clinical outcomes, e.g. heart attack and blood sugar levels, adherence to medication such as prescription filling and persistence, or multiple criteria.[17] In most cases, the detailed conditions and patient health outcome evaluation results remain confidential. A properly constructed PBRSA should include a set of outcomes defining the clinical or economic benefits provided by a therapy for use in a specific population and their measurement methods (data sources, processes and thresholds), providing a way for specifying the net price or reimbursement.[18] Short-term contracts are recommended (18 months to 3 years) to avoid long-term liability and no generic alternatives to ensure value linked to outcomes, not to costs.[19] This implementation should be feasible, taking into account privacy and data availability constraints, and the terms of the contract should include audit and resolution acceptable to both parties.

In a typical PBRSA contract, drug manufacturers provide rebates/discounts to payers, if actual treatment outcomes fail to meet certain criteria (pay-for-failure).[20] However, in practice, PBRSA can also take the form of pay-for-success, in which payers pay less for the success of a treatment, al-

10. See: A. Towse, L. Garrison, *Can't get...*, op. cit., pp. 93–102.

11. J. Adamski, B. Godman, G. Ofierska-Sujkowska, et al., *Correspondence Risk sharing arrangements for pharmaceuticals: potential considerations and recommendations for European payers*, "BMC Health Services Research", 2010, Vol. 10, p. 3, DOI: 10.1186/1472-6963-10-153.

12. The supporters of this division include: M. Wenzl, S. Chapman, *Performance-based managed entry agreements for new medicines in OECD countries and EU member states: How they work and possible improvements going forward*, "OECD Health Working Papers", 2019, No. 115, OECD Publishing, DOI: 10.1787/6e5e4c0f-en; T. Morel, F. Arickx, G. Befrits, et al., *Reconciling uncertainty of costs and outcomes with the need for access to orphan medicinal products: a comparative study of managed entry agreements across seven European countries*," Orphanet Journal of Rare Diseases", 2013, Vol. 8, pp. 1–15, DOI: 10.1186/1750-1172-8-198; L. Coulton, L. Annemans, R. Carter, et al., *Outcomes-based risk-sharing schemes: is there a potential role in the Asia-Pacific markets?*, "Health Outcomes Research in Medicine", 2012, Vol. 3 (4), pp. 205–219, DOI: 10.1016/j.ehrm.2012.07.002; M. Klemp, K.B. Frønsdal, K. Facey, et al., *What principles should govern the use of managed entry agreements?*, "Int J Technol Assess Health Care", 2011, Vol. 27 (1), pp. 77–83, DOI: 10.1017/S0266462310001297; P.P. Barros, *Pharmaceutical policies in European coun-*

lowing it to address the risk of medication adherence at the population level.[21] Success can then refer to a significant improvement in medication adherence for all patients at the end of the period (usually 12 months), based on pharmacy and medical claims data. The marketplace for PBRSAs continues to evolve in individual systems and countries, and will ultimately be judged on their ability to meet the needs of the key players - payers, manufacturers, and patients.

Poland

In Poland, the crisis in access to modern pharmacotherapy is further deepened by the disproportionate growth of the reimbursement budget. The growth rate of the reimbursement budget has been lower than the growth rate of the total NHF benefits budget. For years, expenditure on medicines has not reached the maximum allowed threshold of 17% of the NHF expenditure on benefits. In the NHF 2023 financial plan, the share is approximately 14.61%. The value of the total reimbursement budget is PLN 1.8 billion less than in the 2022 plan. Thus, despite the declared increase in the percentage of GDP on health, as well as an increase in outlays on financing healthcare services, expenditure on medicines has decreased year on year.[22]

The gap between social expectations and public expenditures forced the Polish legislator to turn to RSAs in 2011. They were introduced into the Polish legal system under Art. 11 of the Reimbursement Act,[23] as part of an administrative decision on the inclusion of a medicine in the reimbursement scheme passed by the Ministry of Health. The Polish legislator justified introducing risk-sharing tools by emphasising their potential to reduce public healthcare costs. As a result, they can provide access to new health technologies at a cost that is in alignment with the public payer's capacity. Art. 11.5 proposes several forms of RSA in an open catalogue, mentioning PBRSA in the first place, as "making the applicant's income conditional on the health effects attained."[24]

The process of developing and executing a RSA for a medicine to be eligible for reimbursement starts with a responsible entity (a representative of a manufacturer) submitting an appropriate application (as defined in Art. 24.1.1). As the next step, the application and remaining documentation are submitted to the Agency for Health Technology Assessment and Tariff System (AHT).[25] There, the verification analysis, the position of the Transparency Council and the recommendation of the President of the AHT are prepared.[26] The AHT verifies whether the analyses attached to the application, provided by the Minister of Health, meet the requirements set out in the regulations and evaluates these analyses. It also reviews the reimbursement recommendations for the requested drug, carries out an analysis

tries, "AdvHealth Econ Health Serv Res.", 2010, Vol. 22, pp. 3–27, DOI: 10.1108/s0731-2199(2010)0000022004; J.P. Cook, J.A. Vernon, R. Manning, *Pharmaceutical risk-sharing agreements*, "Pharmacoeconomics", 2008, Vol. 26 (7), pp. 551–556, DOI: 10.2165/00019053-200826070-00002; A. Breckenridge, T. Walley, *Risk sharing and payment by results*, "Clin Pharmacol Ther.", 2008, Vol. 83 (5), pp. 666–667, DOI: 10.1038/clpt.2008.15; J.L. Carapinha, *Setting the stage for risk-sharing agreements: international experiences and outcomes-based reimbursement*, "South African Family Practice", 2008, Vol. 50 (4), pp. 62–65, DOI: 10.1080/20786204.2008.10873741.

13. J. Adamski, B. Godman, G. Ofierska-Sujkowska, et al., *Correspondence Risk...*, op. cit., p. 3.

14. A. Watt, *Risk-sharing...*, op. cit.

15. A detailed breakdown of PBRSA is discussed by A.E. Kim, D.H. Choi, J. Chang, et al., *Performance–Based Risk–Sharing Arrangements (PBRSA): Is it a Solution to Increase Bang for the Buck for Pharmaceutical Reimbursement Strategy for Our Nation and Around the World?*, "Clinical Drug Investigation", 2020, Vol. 40 (12), pp. 1109–1010, DOI: 10.1007/s40261-020-00972-w.

16. J.J. Carlson, K.S. Gries, K. Yeung, et al., *Current status and trends in performance-based risk-sharing arrangements between healthcare payers and medical product manufacturers*, "Appl Health

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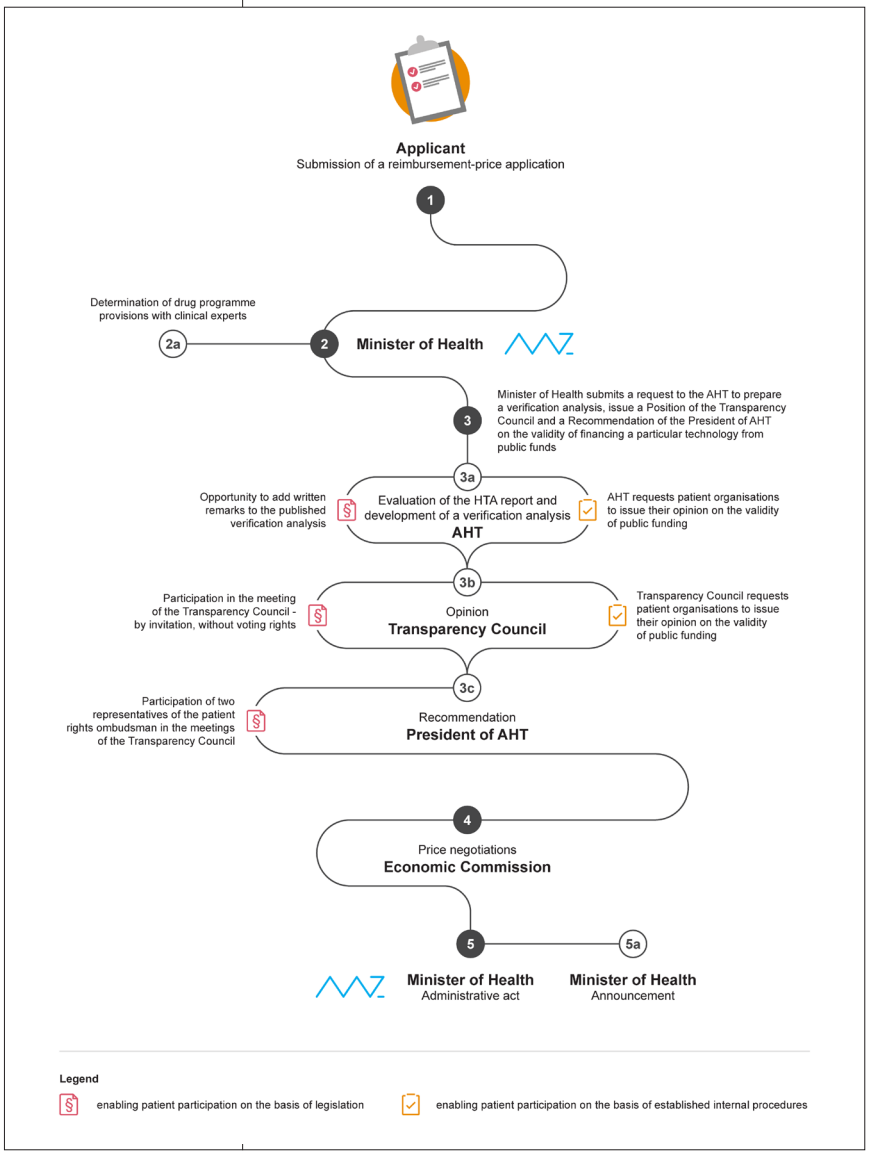


Source: Pacjenci.Pro, *Proces refundacyjny w Polsce*, https://pacjencipro.hta.pl/upload/surveys/364433/files/PacjenciPro_Proces_refundacyjny.pdf, (access 03.08.2023).

of the detailed conditions for reimbursement coverage of the drug, determines the value of the threshold net selling price of the drug and documents all these activities. As part of this process, the AHT assesses the reliability of the drug Health Technology Assessment (HTA) report[27] developed by the drug manufacturer, which consists of four parts: decision problem analysis, clinical analysis, economic analysis, payer budget impact analysis. Then, the documentation is forwarded to the Economic Commission, where the pricing is negotiated with the applicant. This element of negotiation between the authority and the manufacturer regarding RSA, arrangements of which are then included in an administrative decision, suggests a hybrid legal nature, with the norms of administrative and civil law complementing each other.[28] The final decision to include a medicinal product in the list of reimbursed medicines is made by the Minister of Health taking into account the criteria listed in Art. 12 of the Reimbursement Act. These include: the position of the Economic Commission, the recommendation of the President of the AHT, the relevance of the clinical condition to which the application for reimbursement relates, clinical and practical effectiveness, safety of use – taking into account other medical procedures possible in a given clinical condition that can be replaced by the requested medicine, foodstuff for special nutritional use or medical device.[29] The process is represented in Figure 2 below.

Figure 2. Standard reimbursement process for new medicines (which have not been previously reimbursed and have no equivalent)

Econ Health Policy", 2014, Vol. 12 (3), p. 237, DOI: 10.1007/s40258-014-0093-x.

17. N. Pagliarulo, *Pushing 'value,' Harvard Pilgrim tests outcomes deals*, 2018, https://www.biopharmadive.com/news/harvard-pilgrim-astrazeneca-symbicort-value-contract/521659/, (access 07.07.2023).

18. *Value-Based Contracting In The US*, https://www.huronconsultinggroup.com/insights/value-based-contracting-in-us, (access 25.06.2023).

19. Cf. H. Zhang, T. Huang, T. Yan, *A quantitative analysis of risk-sharing agreements with patient support programs for improving medication adherence*, "Health Care Manag Sci.", 2022, Vol 25 (2), pp. 253–274, DOI: 10.1007/s10729-021-09587-9; L.P. Garrison, J.J. Carlson, P.S. Bajaj, et al., *Private sector risk-sharing agreements in the United States: trends, barriers, and prospects*, "Am J Manag Care", 2015, Vol. 21 (9), pp. 632–640.

20. J.T. Kannarkat, Ch.B. Good, E. Kelly, et al., *Examining Misaligned Incentives for Payers and Manufacturers in Value-Based Pharmaceutical Contracts,* "Journal of Managed Care & Specialty Pharmacy", 2020, Vol. 26 (1), pp. 63–66, DOI: 10.18553/jmcp.2020.26.1.63.

21. Ibidem.

22. During the legislative work, the Federation of Polish Entrepreneurs called for funds to be transferred from the

Due to the confidentiality of the negotiations between the applicants and the Minister of Health (represented in this case by the Economic Commission) as to the detailed terms and conditions of the RSAs, it is not possible to analyse individual cases. The last available study analyses 88 reimbursement applications for medicines received by AHT.[30] Among them, there were 55 proposals of RSAs, which accounted for 63% of the total number. As stated by the authors, most applications proposed simple discounts or paybacks and there were no proposed risk sharing instruments classified into category "A" (PBRSAs).[31] The low use of PBRSAs was also confirmed in a report commissioned by the Employers' Union of Innovative Pharmaceutical Companies based on stakeholder interviews conducted in May and June 2018.[32] In the same year, prevalence of simple rebate and payback RSAs was indicated by the President of AHT.[33]

In the case of the PBRSA, following a successful application for infrastructure and organisational preparation, the scheme should be extended to include the design of a reporting process linking billing to the reliability of monitoring the parameters on which the contract settlement depends or the preparation of tools and procedures to deal with the issue of patient data protection. This should be followed by the implementation of a therapy (contracting of services) and billing with the drug manufacturer itself.

Nowadays, the global inflation of the costs of new therapies arouses concerns that the cost of service on the government side may outweigh the savings made on the purchase of medicines. For PBRSA settlement, NHF would need to link benefit billing mechanisms to the submission of data collected in the electronic drug programme monitoring system (SMPT).[34] A major advantage is the modular design of the SMPT, which allows a very flexible definition of the scope of data to be collected and transmitted to the payer and the free establishment of additional functionalities to support the monitoring of treatment in the drug programme. However, each time a dedicated module has to be prepared for a new drug programme, delaying the actual start of patient treatment. In each new instance, the President of the NHF adjusts the SMPT to the new drug programme within 4 months from the publication of the first reimbursement list. On the other hand, the requirement for correct and timely submission of data by the hospital via SMPT applies from the first day of the sixth month following the announcement of the first reimbursement list with this drug programme. Furthermore, key threats to the quality of reported data still seems to be the lack of integration of the SMPT (as a health outcomes monitoring tool) with the billing systems of healthcare providers and the medical records systems of hospitals (HIS),[35] which would allow automatic retrieval of clini-

NHF reserve fund to increase financing for reimbursable therapies.

23. Ustawa z dnia 12 maja 2011 r. o refundacji leków, środków spożywczych specjalnego przeznaczenia żywieniowego oraz wyrobów medycznych, Dz.U. 2011 nr 122 poz. 696 z późn. zm., [Act of 12 May 2011 on the reimbursement of medicines, foodstuffs for special nutritional purposes and medical devices, Journal of Laws 2011, No. 122, item 696 as amended].

24. According to the wording of the amendment coming into force on 1.11.2023.

25. AHT analysts work according to the Health Technology Assessment (HTA) Guidelines, see: Health Technology Assessment (HTA), *Wytyczne oceny technologii medycznych*, https://www.aotm.gov.pl/media/2020/07/20160913_Wytyczne_AOTMiT-1.pdf, (access 15.06.2023).

26. Recommendations and opinions are available via: *Biuletyn Informacji Publicznej Agencji Oceny Technologii Medycznych i Taryfikacji*, https://bipold.aotm.gov.pl/index.php/rada-przejrzystosci/5084-wykaz-obowiazujacych-opinii, (access 15.06.2023).

27. Evaluation of the various consequences of the use of a specific medical technology, particularly in specific clinical situations. It is an interdisciplinary field of knowledge involving the use of scientific methods for health policy. It

cal data. The SMPT is integrated with the billing system of the voivodship branches of the NHF in terms of verifying the compatibility of drug administration/dispensing reported in both systems. Under the current reporting system, the manufacturer is not provided with detailed data to assess the effectiveness of the treatment in specific patient cases and therefore has no tools to control the correctness of the billing.

According to the information I was able to obtain directly from the NHF's Department of Drug Management, the possibility of creating network services for data exchange with healthcare providers' IT systems has been analysed, but will not be carried out under the currently functioning SMPT system.[36] This is due to the complex nature of the system, numerous modifications required following changes in drug programs and the decision to start work on a new system. At the same time, NHF confirmed that the technical aspect of system integration was also envisaged as part of the work on the new system.

Italy

The Italian National Health Service (Servizio Sanitario Nazionale) calls its risk-sharing policy "managed entry agreement" (MEA), which is a form of RSA. The MEA is provided through two schemes: performance-based and finance-based. The agency responsible for MEA is the Italian Medicines Agency (Agenzia Italiana del Farmaco, AIFA), which first introduced its policy in July 2006, aiming at improving cost containment and availability for patients. According to AIFA's Medicines Utilisation Monitoring Centre (OsMed) 2022 report "Medicines use in Italy",[37] the adoption of MEA resulted in 195 million EUR of savings for the healthcare system in that year.[38]

The complex management of discount schemes is entirely based on web-registries run by AIFA. The AIFA Monitoring Registers platform is an IT system that allows access to treatment in a homogeneous manner throughout the country through the control of prescription appropriateness. The system, co-managed with the Regions, also allows the planning and use of medicinal products subject to monitoring on the territory, controlling their expenditure.[39] The drug registries, ones of the most advanced in the EU, are intended to track patient eligibility for treatment pathways in order to evaluate real effectiveness and collect epidemiological data. A drug can be added to a Monitoring Register after it has obtained the marketing authorisation, or after its therapeutic indications have been extended. It is sometimes possible for a drug to be added to a Register before it is placed on the market.

draws on knowledge from epidemiology, biostatistics, economics, law and ethics, among others. HTA provides scientific data for making rational decisions on the use and financing of health services. HTA reports focus on clinical effectiveness and cost-effectiveness analyses; they include systematic reviews and economic analyses, often with recommendations on therapeutic or diagnostic options compared.

28. For a broader theoretical discussion on administrative agreements, see: W. Wojturska, *In search of a remedy for public-private partnership (PPP) hospital infrastructure projects in Poland – a comparative law study Part I: Poland*, "Medicine and Law Journal of World Association for Medical Law", 2023, Vol. 42, No. 2, pp. 458–461.

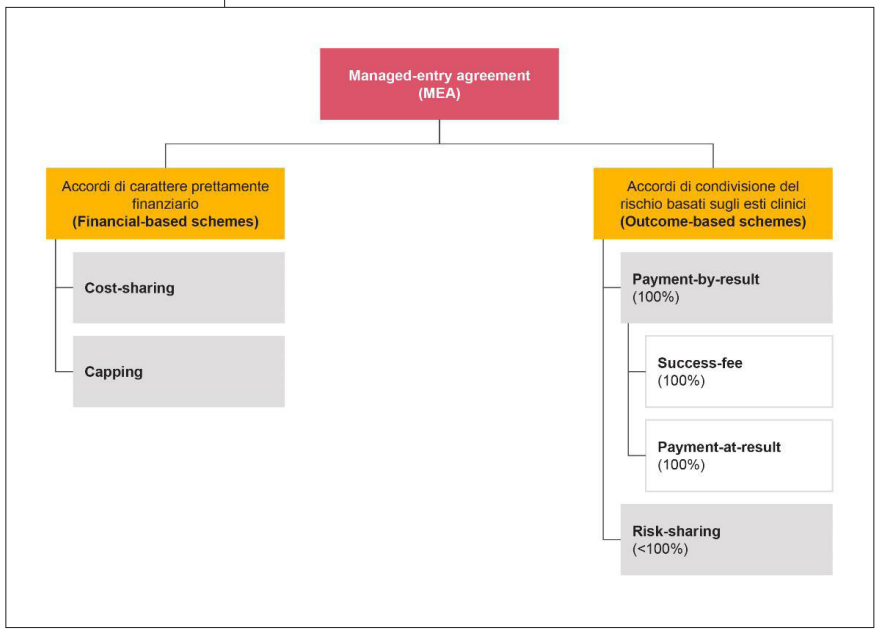
29. M. Klimczak, *Decyzje o refundacji leków są transparentne i podlegają ścisłej kontroli*, 2018, https://www.gov.pl/web/zdrowie/decyzje-o-refundacji-lekow-sa-transparentne-i-podlegaja-scislej-kontroli, (access 30.07.2023).

30. M.K. Pomorski, W. Matusewicz, A. Lipińska, *Types of Risk-Sharing Schemes Proposed in Reimbursement Application Received by AOTMIT in 2015*, "Value in Health", 2016, Vol. 19, https://www.valueinhealthjournal.com/article/S1098-3015(16)32258-6/fulltext, (access 22.06.2023).

31. Cf. P. Kawalec, K.P. Malinowski, *Relating health technology assessment recommendations and reimbursement*

The Regions have significant authority with regard to the AIFA Registers. The Health Directors (Direttori Sanitari) have the authority to grant access to the platform to medical users and pharmacists. Drug prescriptions are issued electronically, including the patient's personal identification, usage and dosage of a drug. A prescription is validated by the system and an e-mail is sent to the hospital pharmacy asking to release the medicine.[40] Based on prescription data, that system monitors the use of innovative specialist drugs according to indications. Additionally, doctors record follow-up clinical data and treatment outcomes. If a patient does not respond to treatment, a hospital pharmacy should apply for reimbursement from the drug manufacturer.

The management of the register platform is organised through a network that includes approximately 3,500 health structures, 52 regional managers, 963 Health Directors, 32,857 doctors and 2,318 pharmacists.[41] Currently, 49 pharmaceutical companies have at least one monitoring register managed by the AIFA platform.[42] Companies interact with individual pharmacies through their profiles on the platform, ensuring that the MEA specified in the negotiations is realised.



Source: AIFA, *L'uso dei...*, op. cit., p. 771.

MEAs are categorized into two types. Payment-by-result (PbR) and Risk-sharing (RS) are classified as PBRSA, while Cost-sharing and Capping are classified as finance-based.

Figure 3. Taxonomy of MEAs from AIFA monitoring registers

In the case of PbR, the entire cost of treatment failure is borne by the pharmaceutical company that owns the drug. This option is typically used if the benefit to risk ratio of a drug presents significant uncertainty. Two variants of PbR are Success-Fee (SF), in which the entire cost of the treatment is borne by the National Health Service (NHS) only when therapeutic success is recorded, and Payment-at-result (PaR), in which the entire cost

decisions in Poland in years 2012–2014, a retrospective analysis, "Health Policy", 2016, Vol. 120 (11), pp. 1240–1248, DOI: 10.1016/j.healthpol.2016.09.021; K. Malinowski, P. Kawalec, W. Trąbka, *Impact of patient outcomes and cost aspects on reimbursement recommendations in Poland in 2012–2014*, "Health Policy", 2016, Vol. 120 (11), pp. 1249–1255, DOI: 10.1016/j.healthpol.2016.09.016.

32. It represents 24 leading pharmaceutical companies engaged in research and development activities and the production of innovative medicines, see: S. Bogusławski, M. Libura, I. Obarska, et al., *Złożone instrumenty dzielenia ryzyka – możliwości rozwoju w Polsce*, 2018,https://www.infarma.pl/assets/files/PEX_Raport_Zloz_instrumenty_dzielenia_ryzyka%E2%80%93mozliwosci_rozwoju_w_Polsce_20190225.pdf, (access 23.06.2023).

33. M. Izmirlieva, *Challenges with risk sharing in Eastern Europe raised at ISPOR Europe meeting*, 2019, https://www.pharmaceutical-technology.com/pricing-and-market-access/challenges-with-risk-sharing-in-eastern-europe-raised-at-ispor-html/, (access 23.06.2023).

34. The SMPT is a system created and updated by the President of the National Health Fund allowing the collection and processing of data on the treatment of patients carried out within the framework of drug programmes with regard to: fulfilment by recipients of the crite-

of the treatment borne by the NHS is spread over time following verification that therapeutic success is maintained. Finally, the second type relates to Risk-sharing (RS), which provides for a discount applicable only to patients who do not respond to treatment. The cost of failure is shared between the NHS and the pharmaceutical company with a variable division, depending on the drug and the pathology (in the event of early treatment failure, the PaR in economic terms actually coincides with a RS model). At the end of 2022, 50% of the total managed entry agreements in place were outcomes-based agreements.[43] Of these, 38% were payment by result and 12% were payment at results.[44] The remaining 50% were finance-based agreement and of these, 23% were cost-sharing and 27% were capping agreements.[45]

The register mainly relate to biological and/or high-cost medicines for the NHS. They cover a number of areas including anti-diabetics, oncology drugs, orphan drugs as well as ophthalmic medicines.[46] A register allows determining indicators that predict the response to treatment and identify patients who can benefit most. AIFA's Scientific Technical advisory Committee (CTS) and the Price and Reimbursement Committee (CPR) select and monitor an appropriate indicator, as well as assess the economic impact of introducing a medicinal product.

Ultimately, the Italian MEA (RSA) system demonstrates the need for communication and collaboration between the various stakeholders, collective involvement in the design of clinical trials, and harmonization of clinical trial procedures. Their indication-based registers appear to successfully support data collection for the purposes of post-launch evaluations and analyses.

United Kingdom

The UK was one of the first countries to implement RSA and is responsible for 56% of all agreements made since 2012.[47] The Pharmaceutical Price Regulation Scheme[48] formally introduced the Patient Access Scheme (PAS) as part of its legal framework in 2009, bringing an important shift in the UK's pricing and reimbursement framework. It allows for the provision of a drug which would not otherwise be supported by NICE and available through the NHS due to insufficient evidence of its cost-effectiveness. The NHS has recently struck a number of large-scale value-based deals and signalled an increased interest in similar agreements.

Companies may submit a PSA proposal for any technology going through the NICE appraisal process. The Patient Access Schemes Liaison Unit (PASLU) reviews and evaluates PAS proposals

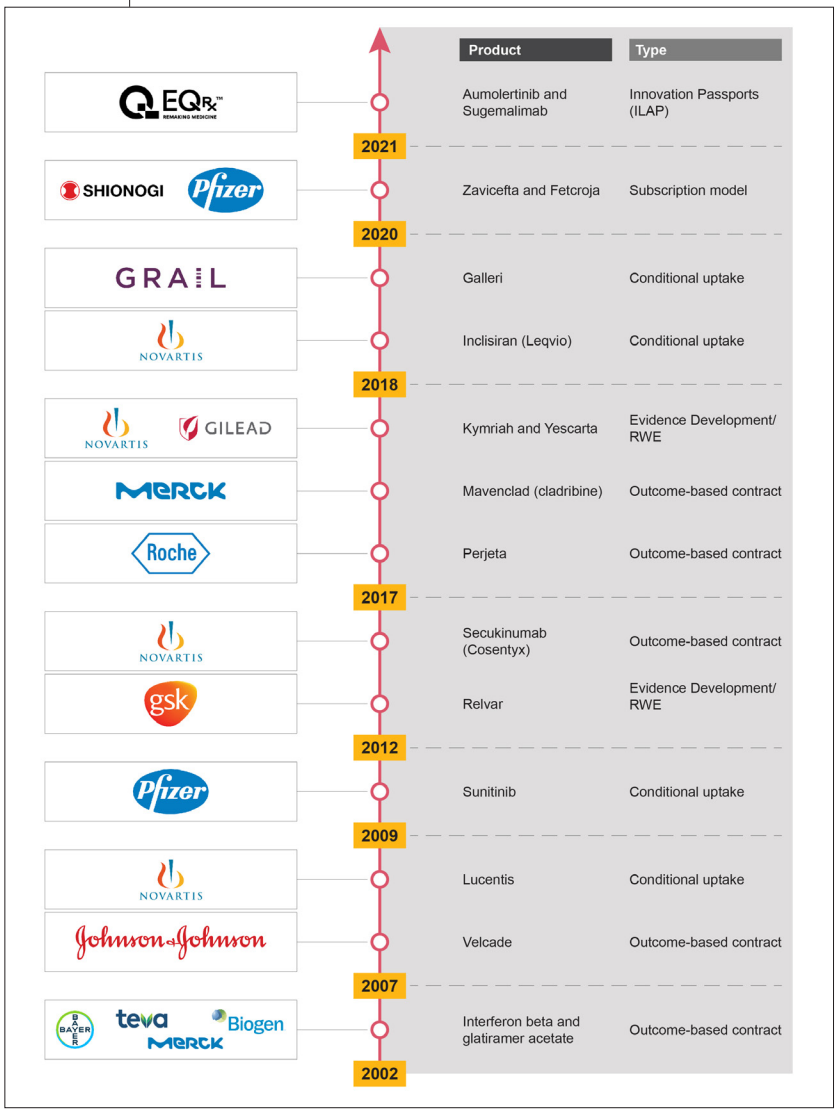
ria for inclusion in the drug programme; qualification of recipients for the drug programme; the therapy applied, including the method of administration and dosage of the drug or foodstuff for special nutritional purposes; the monitoring of the course of treatment and assessment of its efficacy; the date and reason for exclusion from the drug programme; assessment of the effectiveness of the drug programme. A prerequisite for the settlement of the costs of the medicine used in the drug programme and the costs of the related healthcare services is the correct and timely submission of data to the SMPT system.

35. IT system for archiving, processing and sharing data related to the implementation of the diagnostic and therapeutic process.

36. Response from the Director of the NHF Department of Drug Management was submitted 8 November 2023.

37. AIFA, *L'uso dei farmaci in Italia. Rapporto Nazionale Anno 2022*,https://www.aifa.gov.it/documents/20142/1967301/Rapporto-OsMed-2022.pdf, (access 17.08.2023).

38. G. Chirico, *Managed entry agreements: €195m saved by the Italian healthcare system in 2022*, https://remapconsulting.com/managed-entry-agreements/managed-entry-agreements-e195m-saved-by-the-italian-healthcare-system-in-2022/, (access 18.08.2023).



Source: B. Richardson, S. Bentley, P. MacKinnon, et al., *Forming Value-Based Agreements in the UK, 2022*, p. 4, https://www.carnallfarrar.com/wp-content/uploads/2022/05/TL-Value-Based-Agreements_v924.pdf, (access 08.09.2023).

and issues advice to NHS England.[49] Applying companies need to fill out a proposal template, either for a complex scheme or a simple discount scheme. The simple discount scheme involves a fixed pricing agreement that is lower than the list price of the treatment or a percentage discount from the list price, while complex schemes involve performance-based dose caps, rebates or upfront free stock.

Figure 4. Selected examples of VBAs in the UK over time (non-exhaustive)

In 2017, NHS England set a precedent by signing a performance-based deal with Merck for its multiple sclerosis (MS) drug Mavenclad. This made the UK the second country in the world, after Germany, to roll out this drug. The agreement is a good example of PBRSA due to its strong impact on MS care in the UK, as well as the process of generating evidence on the effectiveness and cost-effectiveness of the treatment. Mavenclad's list price was set at over 2000 GBP per 100 mg tablet, which the manufacturer agreed to reduce if the drug did not reach a predetermined level of effectiveness on a sample of patients.[50] While the detailed terms of the agreement were not disclosed, it depended on the outcome data provided by the NHS. The NHS had had problems gathering such data in the past, due to IT infrastructure and data collection issues.[51] Therefore, a success-

39. AIFA, *Registri farmaci sottoposti a monitoraggio*, https://www.aifa.gov.it/web/guest/registri-farmaci-sottoposti-a-monitoraggio (access 06.08.2023).

40. L. Garattini, A. Curto, K. van de Vooren, *Italian risk-sharing agreements on drugs: are they worthwhile?*, "Eur J Health Econ.", 2015, Vol. 16 (1), p. 1, DOI: 10.1007/s10198-014-0585-5.

41. AIFA, *Registri farmaci...*, op. cit.

42. Ibidem.

43. AIFA, *L'uso dei...*, op. cit., p. 772.

44. Ibidem.

45. Ibidem.

46. R. Aggarwal, *Risk-sharing Agreements: Country Experiences and Challenges*, 2014, pp. 1–8, https://www.insead.edu/sites/default/files/assets/dept/centres/hmi/docs/rsa-experiencesand-challenges-insead-hmi.pdf, (access 01.08.2023).

47. The UK is followed by the US, where risk-sharing mechanisms have increased in popularity due to the evolving complexity of the contracts, and by Australia, an early adopter of risk-sharing.

48. As a voluntary agreement between the UK's Department of Health and the branded pharmaceutical industry represented by the Association of the British Pharmaceutical Industry (ABPI), it

ful implementation of the agreement required creating digital data hubs across the country in order to collect the required data. Even though the agreement was highly criticised,[52] it achieved several key benefits. One of the beneficiaries of this deal were specialised MS centres, some of which were newly established at the time of the programme, and have managed to establish a robust network improving in terms of the quantity and quality of services. In addition to changing structural aspects that are important in providing high-quality care to MS patients, the data generated by the programme over a long period of time provide all stakeholders with valuable insights into the disease itself, such as its long-term development, and enable improvements in care.[53] In addition, the mutual risk for the payer and the manufacturer in terms of misdefining the group of patients who may benefit from the drug is reduced.[54]

Another example based on clinical indicators and the presence of clearly defined criteria for success and failure was the agreement recommended in October 2007 for bortezomib (Velcade), a drug for treating a first relapse of multiple myeloma. While the increased survival times with bortezomib had been demonstrated in clinical trials, the NHS was reluctant to make the product available and expressed concern over the value proposition given the price and the informal threshold for valuing QALYs.[55] The manufacturer suggested a risk-sharing plan that amounted to a warranty against relatively poor performance. Under the terms of the NICE recommendation, patients showing a full or partial response to the drug after a maximum of four cycles of treatment, were kept on it, with the treatment funded by the NHS.[56] Patients showing minimal or no response, indicated by a reduction of at least 50% in serum M-protein, would be taken off it and the manufacturer would provide the NHS with either a complete refund or the same amount of product for another patient at no charge.[57]

Lessons learned from previous experience shaped the current direction and priorities at the level of the entire NHS ecosystem that goes toward increasing the likelihood of aligning the ambitions of different stakeholders and successfully agreeing a PBRSA. NHS and NICE have promoted increased access, for instance by accelerating the HTA review, contingent recommendation pending further evidence development, supporting new health technologies and therapies, and several "smart" pricing deals. The NHS has been working to improve its data collection and management practices, with data-driven guidelines becoming more common in partnership. The Integrated Care Systems are another method of improving the planning and delivery of healthcare, allowing new types of pricing innovation and collaboration. The measures described above show the willingness to handle more complex agreements in order to optimise the value for money that is offered to the patients.

regulates the prices of branded prescription medicines and the profits that manufacturers are allowed to make on their sales to the NHS.

49. NICE, *Patient Access Schemes Liaison Unit*, https://www.nice.org.uk/about/what-we-do/patient-access-schemes-liaison-unit#list-of-arrangements, (access 03.09.2023).

50. B. Richardson, S. Bentley, P. MacKinnon, et al., *Forming Value...*, op. cit., p. 5.

51. Ibidem.

52. An initial assessment published in 2009 reviewing patients who started treatment from May 2002 to April 2005 highlighted important methodological issues with this study and the need for longer term follow-up before securing meaningful results. See: M. Boggild, J. Palace, P. Barton, et al., *Multiple sclerosis risk sharing scheme: two year results of clinical cohort study with historical comparator*, "BMJ", 2009, Vol. 339, pp. 1–9, DOI: 10.1136/bmj.b4677.

53. Ibidem.

54. J. Palace, M. Duddy, T. Bregenzer, et al., *Effectiveness and cost-effectiveness of interferon beta and glatiramer acetate in the UK Multiple Sclerosis Risk Sharing Scheme at 6 years: a clinical cohort study with natural history comparator*, "The Lancet Neurology", 2015, Vol. 14 (5), pp. 497–498.

Conclusions

Financial sustainability of health systems is a major concern, given the spiralling healthcare costs caused by investments in fascinating, but still emerging, molecular diagnostic technologies, as well as by the increasing demand for health services, caused mainly by an aging population. As health authorities and pharmaceutical firms are risk-averse, RSA can be seen as a happy medium for ensuring the inherent financial and clinical risks of implementing a new treatment are minimized. Exploring innovative contracting has taken on increased importance due to the extraordinary financial pressure faced by payers in the wake of the COVID-19 pandemic.

PBRSAs address challenges associated with demonstrating the clinical utility of emerging technologies and can potentially reduce the "drug lag" in which patients wait for an unknown amount of time until a particular drug is covered under their health plan. While looking at the experiences elsewhere, the basic lesson to be learned for the Polish healthcare system is that the processing of large data sets has a positive long-term impact on rationalisation of public expenditure, despite its cost-intensiveness (automatic data exchange reduces the level of bureaucracy). Drug manufacturers could offer to set up and fund monitoring registries for outcome-based programmes that integrate with and enhance existing data collection systems, rather than investing in stand-alone drug-specific monitoring projects. The cost of such project might be moderate, if a simple PBRSAs architecture with objective and measurable criteria is used. Only specialised providers should be contracted to provide drug programmes, as the cost of compliance is too high for smaller ones.

The gradual introduction of clinical registers, as well as interoperability of various healthcare systems, will reduce the burden of clinical data entry. A proactive health policy, in which the public side (in particular the Ministry of Health) would create solutions aimed at efficient drug policy spending, is indispensable. It would be beneficial to develop templates of attachments to the reimbursement application by type of RSA, following the example of UK's PAS. Drug manufacturers would benefit from access to anonymised data collected by the NHF, simplifying the development of HTA analyses and proposals for RSA. At the same time, these data would be provided to AHT for the purpose of evaluating the application. The considerations presented in this article can prove valuable for the design of the new SMPT system, in which integration with healthcare providers IT systems is discussed.

55. J. Adamski, B. Godman, G. Ofierska-Sujkowska, et al., *Correspondence Risk...*, op. cit., p. 9.

56. *Cancer-drug refund scheme backed*, http://news.bbc.co.uk/2/hi/6713503.stm, (access 08.09.2023).

57. Ibidem.

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