Conceptual Framework for the Evaluation of Risk Sharing or Patient Access Schemes (RSSs or PASs) in the EU

Noemi Muszbek, MSc, Senior Research Scientist; Zsófia Kiss, MA, MSc, Research Associate; Linda Hortobagyi, MSc, Research Associate, Health Economics and Epidemiology; Boglarka Szegvari, MSc, Associate Director, GMAP, UCB Pharma

OBJECTIVE

The pharmaceutical industry faces constant pressure stemming from healthcare budget restrictions. Within the Organisation for Economic Cooperation and Development (OECD) countries, reductions in public spending on healthcare have in recent years been mainly targeting pharmaceutical budgets. Generic programs, reference pricing, mandated price reductions, and decreased coverage increase the hurdles before market access, contributing to the tension between the supply and demand for drugs. Thus, while keeping in mind budgetary constraints, both policy makers and manufacturers are pressed to find new ways to provide access to patients for new, innovative therapies. Risk-sharing or patient-access schemes (RSSs or PASs) have been suggested as a potential tool for providing access to patients while also decreasing the risks for payers.

RSSs—sometimes also referred to as cost-sharing schemes—can be defined "as agreements concluded by payers and pharmaceutical companies to diminish the impact on payers' budgets for new and existing schemes brought about by uncertainty and/or the need to work within finite budgets."[1]

RSS is not a uniform concept. Its definition is dependent on the type of risks it covers (e.g., financial, uncertainty in safety, efficacy and/or value for money), and the aspect(s) of the drug the scheme is dependent upon (e.g., price, volume, clinical outcomes). Different schemes evolving in different markets are also defined by contrasting terminology.[2,3] This evaluation covers both financial and outcome variations of schemes.

Results from existing RSSs are mixed, and various challenges to a successful implementation have been identified. These include, among others, administrative burden, the cost and infrastructural prerequisite requirements of implementation, follow-up, analysis and assessment, transaction costs, and issues around confidentiality. As a result, RSSs have been frequently labeled as "appealing in theory but hard in practice" and payers tend to prefer simple direct price reductions.[4]

However, direct price reduction, though reducing the cost of new technologies, might compromise efficiency or equity considerations. To overcome the challenges and to ensure the selection of the appropriate RSS, both the constraints of the healthcare environment and the prerequisites of the specific RSS need to be evaluated and taken into account.

A conceptual framework was created to allow the systematic assessment of the countries' relevant factors and the necessary requirements for the successful implementation of specific RSSs, thereby allowing the selection of the most appropriate RSS.

For the development of the framework, oncology was used as an example, as this is the disease area in which RSSs are most frequently discussed, and results are commonly available in the public domain. The rationale behind the most prevalent agreements in oncology can be attributed to the constant increase of the cost of new treatment options, in addition to the well defined endpoints (e.g., progression or level of response

in a relatively short time-frame) that can be evaluated and can serve as the basis for outcome-based agreements. Examples were taken from two European countries—the UK and Hungary—with differing reimbursement systems.

METHODS

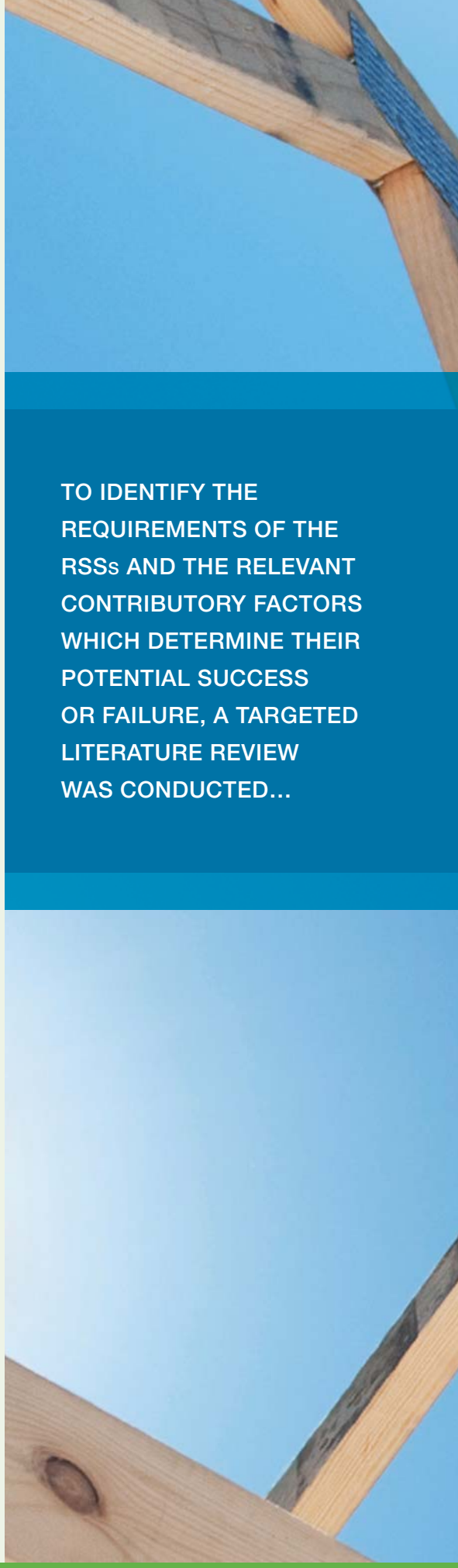
To identify the requirements of the RSSs and the relevant contributory factors which determine their potential success or failure, a targeted literature review was conducted using biomedical databases such as Embase; PubMed; conference proceedings (International Society for Pharmacoeconomics and Outcomes Research [ISPOR]); a relevant Hungarian scientific journal (Informatika és Menedzsment az Egészségügyben [IME]); and Google Scholar to identify and review methodological articles describing risk sharing schemes. Search terms included expressions for RSS (e.g., conditional coverage; conditional reimbursement; risk sharing; coverage with evidence; value-based pricing, pharmaceuticals; risk adjustment scheme; risk adjusted compensation scheme) and different synonyms and related terms of cancer. 57 abstracts were identified; however, after screening abstracts and full texts, only 14 were considered relevant, the majority analyzing RSSs from the UK.

Based on the targeted review and the evaluation of the currently implemented RSSs in the target countries (UK and Hungary), a three-level conceptual framework was constructed. The requirements of current and planned RSSs, and contributory factors determining their success, were extracted from the literature and were structured according to the Political, Economic, Social and Technological (PEST) criteria analysis framework. For each criteria a list of requirements that needed to be met in order to fulfill each criterion were identified.

The list of requirements was transformed into a questionnaire with close-ended answers. The potential answers were determined with the help of an ordinal-level rating scale (1–3, with 1 standing for requirements not met, and 3 for requirements fully met) and the questions were organized by the criteria. Both RSSs and countries can be evaluated using this simple scoring system for each criterion. To narrow the focus of the study, criteria and RSSs relevant for drugs in oncology were taken into account.

The questionnaires were validated by two EU industry experts and one payer's representative from the UK and Hungary. The validated questionnaire was completed for the UK and Hungary and for the following four RSSs in oncology:

1. **Direct Price Reduction** is defined as an agreed percentage (e.g., 10%) price reduction from the price of the drug. This reduction is paid back by the manufacturer to the payer at the end of a given period based on the number of administrations.
2. **Indirect Price Reduction or "Special Offers"** involve offering certain packs/vials for free. The scheme evaluated is when the manufacturer provides first pack/administration of the treatment for free, i.e., the price of the first pack is paid back by the manufacturer to the payer at the end of a given period based on the number of newly treated patients.
3. **Outcome-based Scheme or Outcome Guarantee** or "No cure, no pay" schemes are based on a predefined clinical performance criterion. For example, if a patient progresses within a given time period (e.g., three months) of treatment initiation, the cost of treatment will be paid back by the manufacturer to the payer at the end of this time period, based on the number of newly progressed patients.



TO IDENTIFY THE REQUIREMENTS OF THE RSSs AND THE RELEVANT CONTRIBUTORY FACTORS WHICH DETERMINE THEIR POTENTIAL SUCCESS OR FAILURE, A TARGETED LITERATURE REVIEW WAS CONDUCTED...

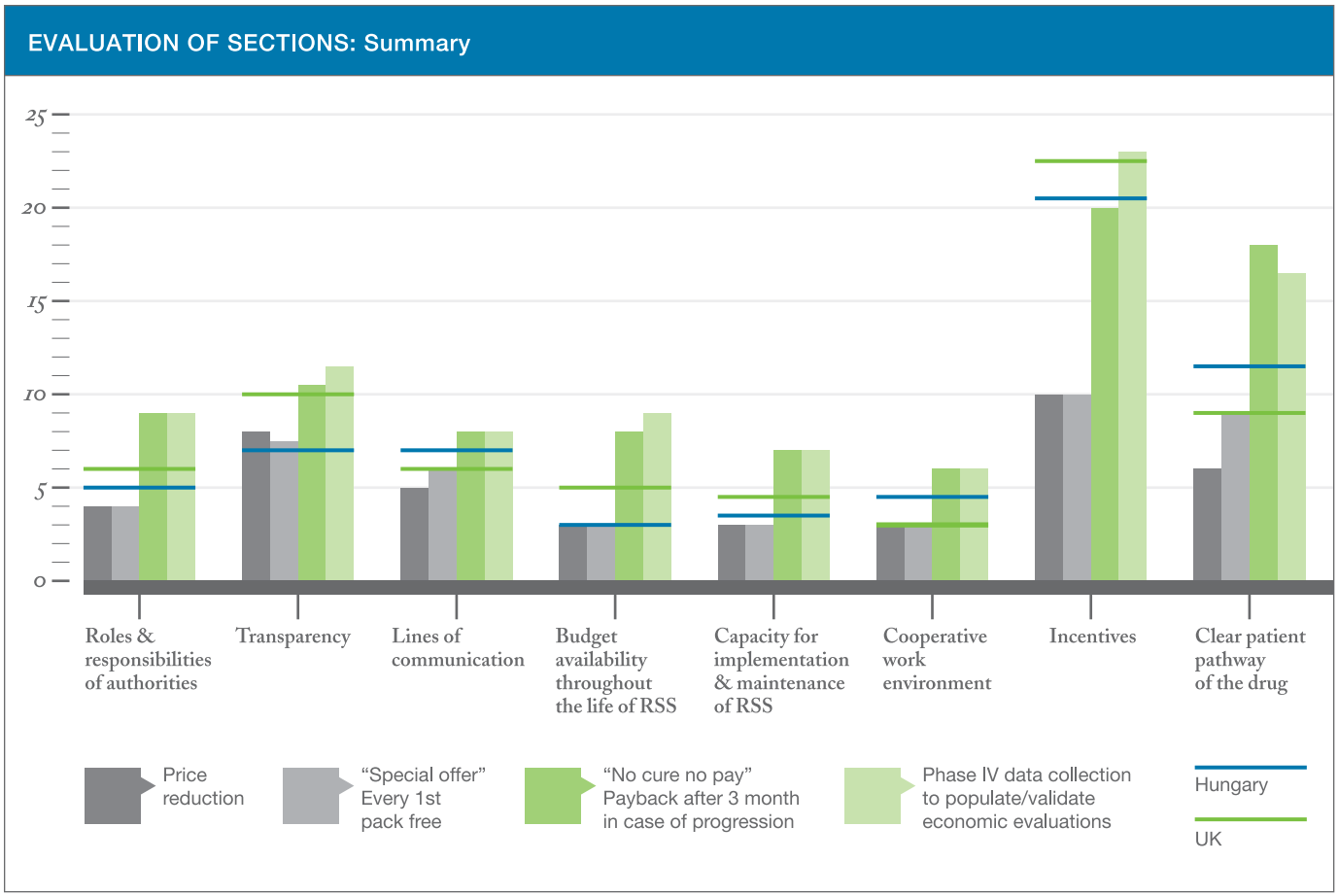


figure 1

4. **Outcome-based Scheme or Uncertainty Reduction** refers to phase IV data collection to populate or validate economic evaluations to reduce the uncertainty of the cost-effectiveness results. In this example the manufacturer initiates a study, collects the data and provides the information to the payer. If with the new data the revised ICER exceeds the threshold, the cost of drug is reduced so that the threshold is met.

By analyzing the results according to the requirements or criteria, the possibility of success of the different RSSs in a given country could be evaluated. The minimum requirements for a successful RSS according to a given criteria and/or given requirement were plotted against the country's performance in the given area. When RSS scores were lower, or at the level of the country's performance in a given requirement/criterion, the RSS was considered a "good fit" for that country in that respect. As the criteria have a differing number of requirements, and their relative importance can vary, the summary scores cannot be estimated.

RESULTS

Questionnaire

Based on the findings of the targeted literature review, 38 requirements were identified and grouped according to eight criteria. These criteria were:

- **Roles and responsibilities of the authorities** incorporating questions regarding the legal framework in place in a given country and the roles and responsibilities of the pricing and reimbursement bodies and manufacturers;
- **Transparency** of the decision making process (e.g., requirements and decision criteria during the negotiations of the market access process), results and follow-up;
- **Lines of communication** as an element of the process;
- **Availability of budget or potential for additional funds** for the implementation of the RSS and follow-up of RSSs;

- **Capacity for implementation and maintenance** of the RSS in terms of staff and equipment;
- The environment for the presence of **trust and cooperation** between the stakeholders in the different phases of an RSS (design, implementation and evaluation);
- **Incentives** for the different stakeholders (decision maker authorities, healthcare system providers, healthcare professionals, population) to understand the level of engagement of each party involved;
- **Clear patient pathways** are available or can be defined, monitored, and feedback can be provided.

Assessment of RSSs

The evaluation of the criteria for the four sample RSSs showed that although the requirements to implement a successful outcomes-based scheme are higher than for a price reduction schemes, the importance of transparency and the availability of clear and appropriate lines of communication are similar between the simpler and the more complex schemes *(Figure 1)*. Thus the following are essential elements of even the simplest scheme:

- The accessibility of the market access requirements and decision criteria to all stakeholders
- The transparency of the market access negotiation process
- The possibility of commercial confidentiality in relation to the terms of the RSS
- The openness of communication between the different stakeholders

To ensure the success of the more complex outcome-based schemes compared to the simpler price reduction schemes, the right incentive system for stakeholders, clear patient pathways, and the appropriate budgetary and capacity provisions require additional attention prior to the implementation. In both the UK and Hungary, the simpler price

REQUIREMENTS OF A SUCCESSFUL RSS: "Roles and Responsibilities of Authorities" and "Transparency"—Hungary (criteria plotted against the country's performance on a 1–3 scale)

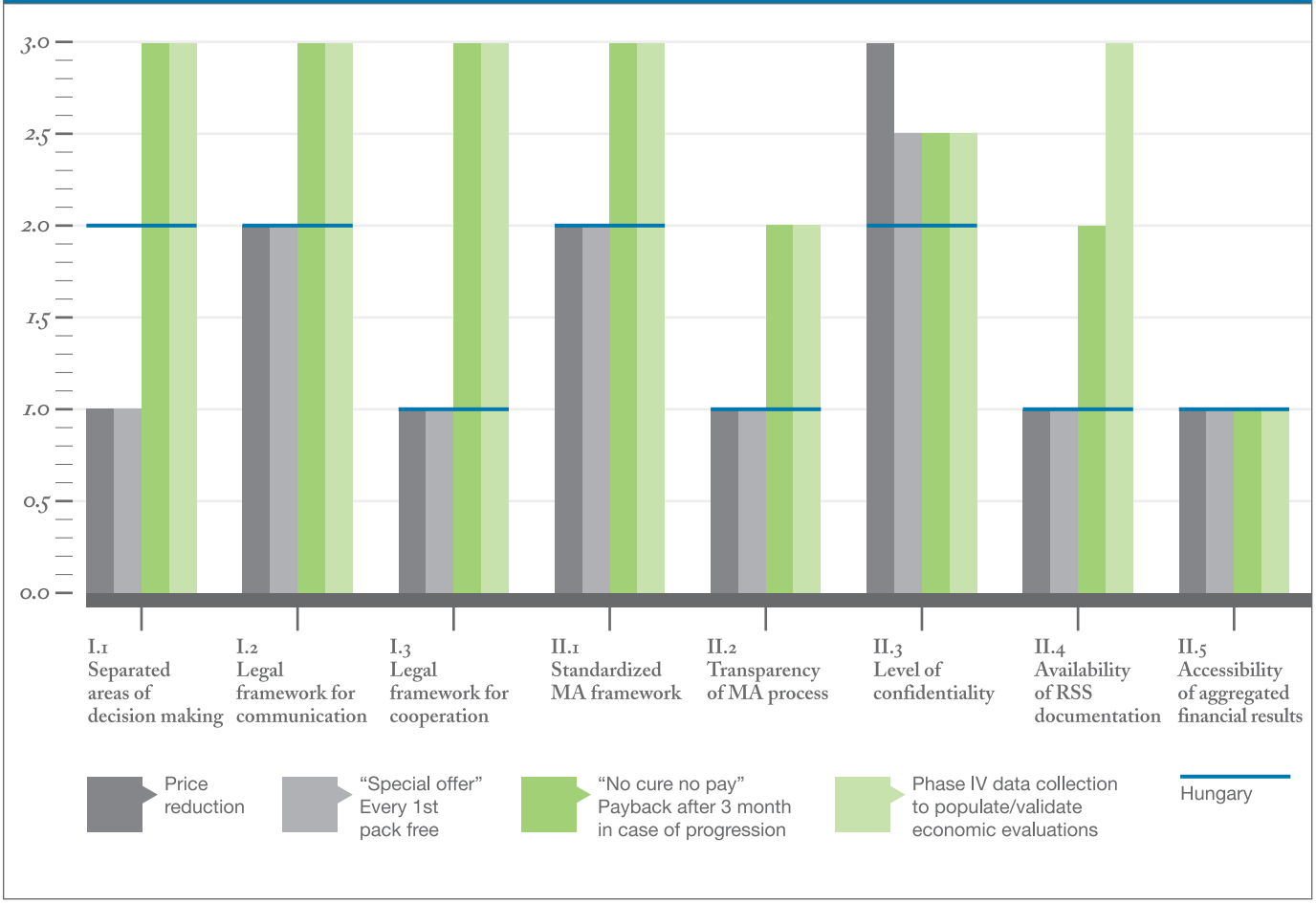


figure 2

reduction schemes have a better chance of success, with different criteria requiring additional attention for the more complex schemes. To evaluate these criteria, and the potential changes required for a successful RSS, the individual requirements need to be assessed.

For example, while in Hungary the accessibility of aggregate financial results is sufficient to implement and assess more complex schemes, the level of confidentiality could hinder the implementation of even the optimal level of simpler price reduction schemes *(Figure 2)*. At the same time, since in Hungary the stakeholders' expectations of the decision-making process are based on evaluating prior decisions, the legal framework encourages the use of the simpler schemes and creates too much uncertainty for the more complex schemes.

Contrastingly, in the UK, while the system has the potential to handle the additional burden of both the simpler schemes and the "no cure no pay" type of RSSs, the implementation of phase IV data collection requires additional funding *(Figure 3)*. To fulfill the infrastructural requirements of the outcomes-based schemes, additional investment is needed from the stakeholders. However, even with sufficient level of investment and additional funding within the criteria of "work environment", the collaboration between the different stakeholders can currently significantly hinder the implementation of successful outcomes-based schemes. Similarly the availability of trust and willingness is not perceived sufficient for these types of schemes.

DISCUSSION AND CONCLUSION

To aid in the selection of the most appropriate RSS in a given healthcare environment, to assess the potential for an RSS, and to identify the gaps in the healthcare system that could jeopardize a successful implementation, a conceptual framework was developed. Via the simple scoring system, the criteria level assessment allows the evaluation of the requirements of different RSSs, in contrast to the possibilities within a given healthcare environment. At the same time, the requirement level assessment facilitates a more richly-detailed review of why a specific RSS would potentially be successful or not, and what steps would be required to ensure success.

Through these assessments, the framework could aid both decision-making processes and the strategic planning of stakeholders. For regulators, a systematic assessment of the potential RSSs, and the steps required for their successful implementation and follow-up, could aid budgetary planning and improve patients' access to new, innovative medicines.

RSSs are also an important part of market access strategies developed early in the product lifecycle. The target product profile already might suggest the possible directions in terms of a given compound/indication, helping to assess the RSS that would suit the product. However, the applicability and impact of the RSSs vary greatly between countries, requiring a complex, country-specific assessment of the healthcare system. Currently if done, this assessment is mostly based on past experience and precedents, while decisions are made in response to regulatory decisions, requests, and health technology assessments. This, in many cases, results in simple price reduction schemes with its set of consequences regarding global pricing strategy. Hitherto, assessments were reactive and reached for the broadest solution; this new approach allows the possibility to systematically evaluate each healthcare environment and the reasonable RSSs, and could be beneficial in finding the best fit to the product strategy.

The conceptual framework discussed here could provide the required structured insight into given healthcare settings, and would be able to identify the aspects of the environment that need to be challenged, shaped, and improved for a better outcome. This could improve the efficiency of the healthcare system, patient access, and the goal of meeting budgetary constraints. Suboptimal or failed schemes result in unnecessary expenditure, providing inadequate value for money.

This framework is currently experimental with various limitations. The different criteria can only be evaluated independently from each other, i.e, there is no overall summary score to assess the overall performance of the RSSs and countries as there is no information on the relative weight of the criteria. Similarly, since the scores are only on an ordinal level scale, the relative difference between the schemes or countries cannot be assessed. For example, results cannot describe

how much worse the environment is for one RSS compared to the other—only that it is worse. Although the summary score would only help in providing yes/no answers, adding relative weights to the criteria would also help in prioritizing the steps needed to ensure a successful RSS.

For the validation exercise, only one payer and one industry expert was interviewed per country. A more reliable assessment would be provided by conducting more extensive payer research to provide input for the framework. Similarly, the RSSs used for validation are textbook examples. The evaluation of currently used RSSs is required to aid decision making. In addition, the inclusion of further countries and additional therapeutic areas into the framework would offer greater flexibility.

Although it is still in development, the conceptual framework offers a good starting point for the evaluation of the potential success of the different RSSs in oncology in a given country.

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REQUIREMENTS OF A SUCCESSFUL RSS: "Capacity for Implementation and Maintenance of RSS" and "Work Environment"—UK (criteria plotted against the country's performance on a 1–3 scale)

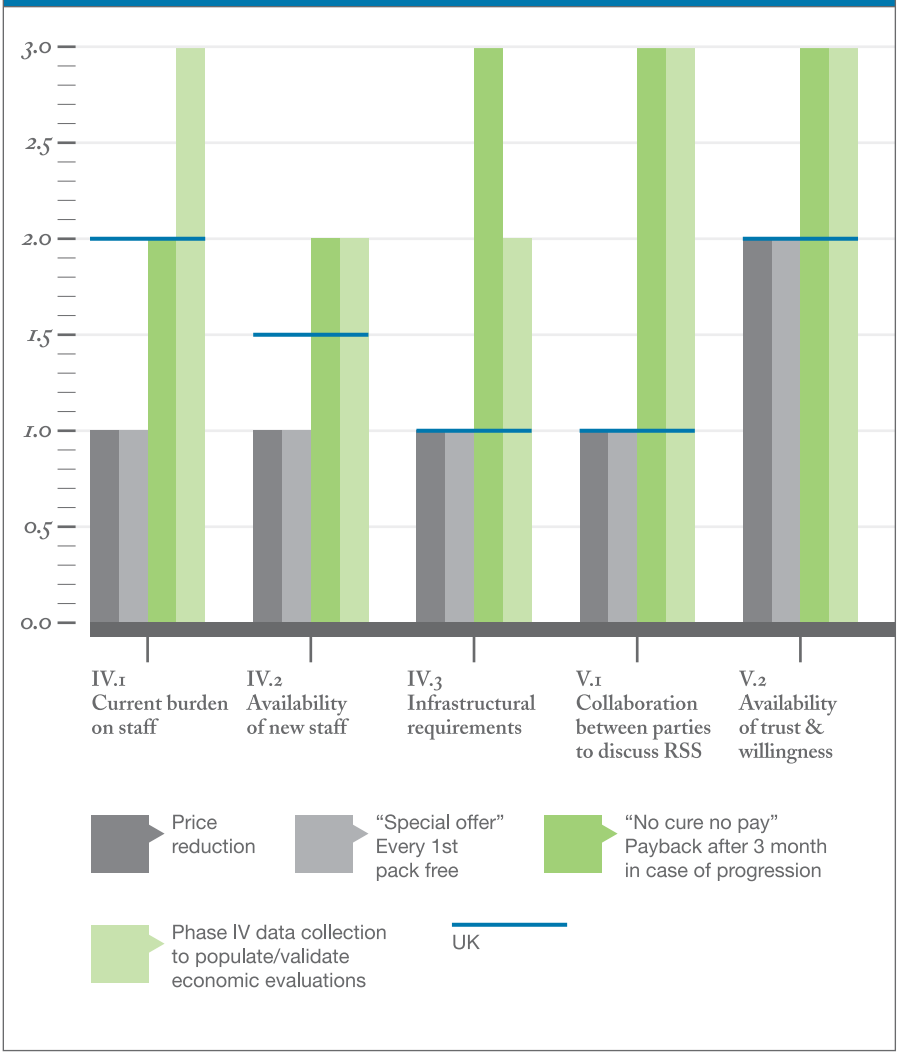

figure 3

For more information, contact Noemi.Muszbek@evidera.com, Zsofia.Kiss@evidera.com or Linda.Hortobagyi@evidera.com.

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