Improving the use of real-world evidence from managed access: the Systemic Anti‑Cancer Therapy data set

HTA Innovation Laboratory report

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# Executive summary

This report presents the findings of a Health Technology Assessment Innovation Laboratory (HTA Lab) project exploring the use of real-world evidence from the Systemic Anti-Cancer Therapy (SACT) dataset to inform NICE re-evaluations of cancer treatments exiting the Cancer Drugs Fund (CDF). Although SACT data is routinely collected during managed access, there is no consistent approach to using this data to inform cost-effectiveness analyses and committee decision making.

To address this gap, the NICE Decision Support Unit (DSU) was commissioned to expand on a pilot project that examined 4 technology appraisals after CDF exit. The DSU then applied a structured modelling approach to 8 further re-evaluations that used SACT data. It found that overall survival was often lower and time on treatment shorter in the real-world data (RWD) compared with trial data. By incorporating these differences into economic models through scenario analyses, the DSU demonstrated that using SACT-based results is feasible and can be informative.

This report makes recommendations for how NICE can make more consistent and meaningful use of SACT data. These include (1) setting clear expectations for companies to routinely assess the feasibility of providing SACT-based scenarios at re-evaluation, (2) improving guidance and training for technical staff and committees, and (3) developing methods for using RWD when comparator information is limited. To support implementation, the recommendations are grouped into: immediate implications for NICE, which are presented in the main report, and suggestions to enhance the way SACT data is collected and used, which are set out in the appendix.

# Background

As described in the [NICE strategy 2021 to 2026 (PDF only)](https://static.nice.org.uk/NICE%20strategy%202021%20to%202026%20-%20Dynamic,%20Collaborative,%20Excellent.pdf), NICE would like to use RWD to resolve gaps in knowledge and improve access to innovations for patients. In support of this goal, the HTA Lab has explored how data collected during managed access can better inform decision making.

When a cancer treatment is recommended for use within the CDF, RWD on overall survival and time on treatment can be collected via the national SACT data set. This aims to reduce uncertainty around key parameters that affect cost effectiveness and sometimes clinical effectiveness. But despite routine SACT data collection and ongoing investment in managed access, this data is rarely used to update the economic model at re-evaluation (even when it may have been feasible to do so), and there is no clear guidance on when or how to incorporate it.

At the time of writing, SACT data is typically only available for the new intervention. Although there may be relevant comparator data in the dataset, it is not routinely made available or analysed as part of the re-evaluation process. This limits the ability to directly observe real-world relative effectiveness and creates uncertainty in how to model comparative outcomes.

To address this gap, the HTA Lab has worked with the DSU to assess whether SACT data could feasibly be used to generate cost-effectiveness estimates at the point of re-evaluation, and what the implications of doing so would be for NICE’s methods, processes, and decision making.

This report summarises the work undertaken by the HTA Lab to date and outlines the implications for NICE’s approach to using RWD in re-evaluations of cancer technologies.

# The rationale for this report

There is growing recognition that real-world evidence (RWE) can play a vital role in improving the relevance and credibility of NICE decisions, particularly for technologies that have been available through managed access. But the evaluation committees lack clear guidance on whether and how to use the SACT data presented at re-evaluation, leading to inconsistent practice and potentially valuable information being underused.

This project responds to concerns from NICE committees, technical staff, and stakeholders that SACT data is not being used to its full potential. A small number of evaluations have explored SACT-based incremental cost-effectiveness ratios (ICERs) using bespoke methods, but there has been no standardised or endorsed approach.

There is no formal guidance, or expectations, about how evidence review groups or committees should model or interpret SACT-based evidence in cost-effectiveness analysis. This contributes to inconsistent practice across evaluations.

By evaluating a structured and reproducible method for incorporating SACT data into cost-effectiveness models, this HTA Lab project aimed to:

* determine when this data might meaningfully inform re-evaluation decisions
* assess the practical feasibility of using this approach within current NICE processes
* explore what further action is needed to ensure that managed access data collection delivers maximum value to the health and care system.

The findings will help NICE decide whether, and in what circumstances, committees should use SACT-based analyses to support re-evaluation, and how this might be reflected in future guidance, processes, or methodological updates.

# Scoping work

In April 2024, the DSU delivered a report to NICE exploring how RWD from the SACT dataset could be used in cost-effectiveness analyses during re-evaluations of technologies exiting the CDF. The report focused on 4 case study technology appraisals in which aggregate-level SACT data indicated that outcomes in routine NHS practice differed from those seen in the corresponding clinical trials1, 2, 3, 4. It explored methods to capture the SACT data at the managed access re-evaluation stage and concluded that detailed examination of more case studies with different characteristics would be valuable. This could lead to the development of a framework to guide when using SACT data is most likely to be materially important to NICE committee decision making (for example, based on characteristics of the condition or data). It could also lead to clearer insights into which methods are most appropriate for such analyses, with and without real-world comparator data.

# DSU report

Building on the scoping work, the DSU did a follow-on analysis to explore the feasibility and value of using SACT data across a broader sample of case studies, particularly in cases where real-world outcomes differed from trial results5.

The DSU selected 8 case study technology appraisals that met predefined selection criteria6, 7, 8, 9, 10, 11, 12, 13. These included:

* treatments with sufficient SACT sample sizes for meaningful analysis, except in one ‘low sample size’ complex case
* cases in which SACT outcomes (overall survival and time on treatment) were available and appeared to diverge from the trial outcomes, but were not key to the re-evaluation committee’s decision
* economic models that were suitable for re-analysis using the SACT evidence
* a variety of decision problem complexities, such as having a one-time therapy and treatments with stopping rules.

Across all of the case studies, SACT data was only available for the intervention, not the comparator; this is typical of the current NICE re-evaluation process. The DSU addressed this by applying relative treatment effects from the original trial to the SACT data, allowing it to model pseudo real-world outcomes for the comparator arm.

Key findings included:

* SACT-based overall survival was typically lower than trial-based survival; time on treatment was often shorter as well.
* In some cases, worse overall survival was offset by shorter time on treatment, resulting in relatively stable ICERs. In others, particularly where survival was reduced but treatment costs remained similar, the ICER increased substantially. SACT-based ICERs were at least 20% higher than trial-based ICERs in 5 cases, and was 199% higher in 1 case. In 4 evaluations the ICER changed by less than 5%, and in 2 cases the ICER decreased, most notably by 22% in the [NICE technology appraisal of tisagenlecleucel for treating relapsed or refractory B-cell acute lymphoblastic leukaemia](https://www.nice.org.uk/guidance/ta975).
* In 5 of the 11 technology appraisals reviewed (including those from earlier pilot work), the SACT-based ICERs were judged likely to have influenced committee decision making if they had been available.
* The DSU concluded that although SACT data may not always be suitable for use as a base-case analysis, it is well suited to scenario analyses, particularly when there is uncertainty around the external validity of trial data.

The DSU report also outlined 3 typical scenarios in which SACT-based analyses may be most informative:

* Scenario 1: SACT and trial overall survival outcomes are similar, but SACT time on treatment is different from that in the trial. Here, the DSU advised that SACT-based time on treatment could be used in the model. This is typically feasible because of the observed maturity of real-world time on treatment data.
* Scenario 2: Overall survival outcomes in the SACT data are different from the trial, but time on treatment outcomes are similar. Here, the DSU advised re-running the economic model using either trial-based or SACT-based estimates of time on treatment, because they are similar anyway, and fitting new survival curves to the SACT overall survival data to reflect real-world outcomes.
* Scenario 3: Both overall survival and time on treatment outcomes are different in the SACT data than the trial. Here, the DSU advised updating the model using SACT-based time on treatment and fitting new survival curves to the SACT overall survival data. This is the most complex scenario to model but remains feasible.

The DSU’s approach reflected what is currently feasible using the data typically available at re-evaluation. While pragmatic and reproducible, the analysis has several limitations. It assumes that the relative treatment effect observed in the trial can be applied to real-world outcomes, and it does not include progression-free survival, which is not available in the SACT dataset. The analysis also lacks a real-world comparator arm and relies on shorter follow up compared with trial data. These limitations underpin the rationale for applying adjusted estimates of survival rather than directly modelling from SACT data alone.

Overall, the DSU concluded that SACT-based scenario analyses could be done routinely and provide committees with valuable additional insight into the real-world performance of cancer treatments. While data limitations remain, including the lack of real-world comparator and progression-free survival data, the methods demonstrated in the report are feasible to implement and, in just under half of the cases studied, materially relevant to NICE’s decision making.

The DSU recommended that these analyses could support NICE committee decision making in the following ways:

* As a scenario analysis, particularly where SACT data suggests different survival or different time on treatment compared with trial populations.
* To inform estimates of likely ICER ranges, helping committees understand how results might vary under real-world conditions.
* To supplement trial evidence, especially if trial data is immature or not fully generalisable to NHS populations.
* To ensure that managed access data is used meaningfully, adding value to the data collection already built into the CDF process.

These insights form the basis for the immediate recommendations set out in the following section, with further suggestions included in Appendix 1.

# Recommendations

The findings of this HTA Lab project, informed by the DSU’s technical work and internal stakeholder engagement, highlight a clear opportunity for NICE to make more meaningful use of data collected through managed access. This section gives recommendations that are focused on immediate implications for NICE (that is, actions NICE should take now using the SACT data typically available at re-evaluation), and that are operationally feasible.

Additional suggestions for future methodological development and data infrastructure improvement are provided in Appendix 1, covering:

* advancing the use of SACT data using existing methods and infrastructure
* opportunities to enhance the SACT dataset to support future decision making.

## Immediate implications for NICE

The HTA Lab recommends that, when feasible, NICE introduces SACT-based scenario analyses as standard during cancer treatment re-evaluations. This should be supported by the actions listed below.

|  |
| --- |
| 1. Companies should routinely assess the feasibility of including SACT-based scenario analyses in re-evaluation submissions. In particular, companies should consider applying the approach tested by the DSU, using SACT data for the intervention and the relative treatment effect from the trial to generate a pseudo-comparator arm. These analyses would enable committees to explore real-world outcomes in a structured and consistent way and ensure better use of data collected through the CDF. 2. NICE should clearly set the expectation that companies include SACT-based scenario analyses in all future re-evaluation submissions, particularly if the necessary aggregate SACT data is available and of sufficient quality (and with agreement with all parties in the managed access agreement). These scenarios should use the company’s submitted model and be included routinely, when feasible. 3. If SACT data is not used, companies should justify this clearly. This should only be done in exceptional circumstances (for example, if aggregated SACT data was not made available to the company or NICE, or are of insufficient quality to use, or show no discernible differences in outcomes compared with the trial data). 4. NICE should provide its technical staff, committee members, and companies with practical guidance on how to interpret SACT-based scenarios, and what questions to ask when results diverge from trial-based expectations. To this end, the HTA Lab will produce a single-page summary explaining the DSU approach at a glance, for the benefit of internal colleagues and committee members. The DSU report, which has been published on the DSU’s website, provides in-depth technical guidance, which will be particularly appropriate for external assessment groups and companies. 5. NICE technical teams and committee chairs should encourage committee members to consider the SACT-based scenario analyses when evaluating the level of ICER uncertainty, particularly when survival outcomes and treatment duration change commensurately. While this may result in a similar ICER, it could indicate important differences in the real world beyond overall survival and time on treatment (in, for example, treatment effectiveness, patient characteristics, or progression patterns) that merit further scrutiny. |

## Additional considerations

To support consistent implementation, NICE should consider the following practicalities:

* Because the CDF is a joint partnership with NHS England (NHSE) who is also the data controller for SACT, NHSE would need to be consulted on any changes relating to ongoing managed access agreements. But the recommended DSU approach does not change how SACT data is collected or provided. For ease, we recommend that the requirement for routine consideration of the feasibility of SACT-based analyses is included in all future managed access agreements. For any re-evaluations as part of ongoing managed access agreements, the DSU report and this HTA Lab report will give committee members and all stakeholders sufficient opportunity to request and provide the relevant analyses, when feasible.
* NICE’s technical and managed access teams should regularly consider whether SACT-based results are being presented clearly to committees, and encourage clear documentation of methodological choices. This will support reproducibility and facilitate committee interpretation. Presentation could include summaries of ICER ranges or visual indicators showing model sensitivity to using SACT data. Transparent documentation could include descriptions of the clinical codes, cohort definitions, and any assumptions used in the SACT-based analyses.

# Conclusion

This HTA Lab project has shown that SACT data can add meaningful value to NICE re-evaluation for treatments in managed access. The DSU's analysis has demonstrated that RWE from managed access can be incorporated into cost-effectiveness models, when aggregate SACT data are available and of sufficient quality, by applying unbiased trial-derived estimates of relative effectiveness to real-world overall survival and time on treatment outcomes for the new technology to construct pseudo real-world comparator outcomes5. These analyses can expose potential risks from relying solely on trial-based evidence and offer committees a more complete picture of how treatments may perform in routine NHS practice relative to current practice.

The SACT data may have limitations that mean NICE committees prefer to place substantial weight on trial-based analyses in their decision-making. But the DSU’s approach shows that simple scenario analyses can help to characterise the decision uncertainty more fully, particularly when survival outcomes or treatment durations differ between trial and real-world settings. In both cases, these analyses can provide additional assurance about the robustness or generalisability of the trial evidence. They should be done routinely (when feasible) to provide additional data points in the range of plausible ICERs that committees consider in their decision making. This will allow for more informed judgement, supports the credibility of NICE guidance and methodology, and ensures that data collected through managed access arrangements are used meaningfully.

The recommendations and suggestions in this report provide a clear and practical path forward. NICE, in partnership with NHSE, can act now on the immediate implications by:

* setting expectations for routinely including SACT-based scenarios in company submissions
* providing guidance and training for staff and committees
* improving how results are interpreted and presented.

Further methodological enhancements and improved data access would help NICE to make better use of existing SACT data and support the use of RWE more broadly (see Appendix 1).

This work highlights not only the feasibility of this approach, but also the opportunity for NICE to align its processes with its commitment to using RWE to reduce uncertainty and support timely, evidence-informed access to promising technologies throughout their lifecycle.

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# Appendix 1: Suggestions for advancing the use and collection of SACT data

The recommendations contained in this HTA Lab report outline an opportunity for NICE to make better use of aggregate-level SACT data on overall survival and time on treatment that is currently made routinely available at re-evaluation. As part of this project, the HTA Lab working group thought that there may be further potential methodological and process developments that could:

* help NICE make better use of the SACT data already being collected, which would build on the approach explored in the DSU analysis
* improve SACT data infrastructure to enhance its value and usability for NICE decision making.

Suggestions to support these ends are listed below and should be considered in future work to advance the use of RWE by NICE, NHSE and stakeholders; however, they are not immediate recommendations for action.

## Suggestions to advance the use of SACT data

* Develop technical guidance on how to incorporate SACT-based survival insights into trial-based survival curves by applying a factor to reduce absolute survival, while retaining the original curve shape and the benefit of longer follow up in the trial data. The DSU proposed this approach in scenarios 2 and 3 as a more reliable alternative to fitting new survival models directly to SACT data, which often have limited follow up and small sample sizes. Applying such a factor enables the use of RWE in cost-effectiveness modelling, while preserving the structural and comparative validity of trial-based estimates. The DSU contract could be used to enable the DSU to do this work as an addendum to their report for this project.
* Support realistic real-world comparisons using SACT data. In many cases, where the comparator is a systemic anticancer therapy, relevant historic data is held in the SACT data set. Although fewer patients may have treatment with the comparator after the intervention becomes available through the CDF, historic data with longer follow up is likely to exist. Making this data more accessible at re-evaluation could help reduce reliance on assumptions and improve the validity of comparisons. However, there are practical challenges, including an agreed definition of a relevant historic comparator group, and timing mismatch between older comparator data and more recent intervention data.
* Embed the use of SACT data within a lifecycle approach to health technology assessment, aligning with NICE’s wider strategic objectives. Rather than relying on fixed timelines for re-evaluation (for example, after 3 years), a more flexible model could allow guidance to be reviewed when sufficient RWD has been collected. This might include predefined criteria for minimum data maturity, trigger points for extending data collection, or reintroducing a technology into managed access if critical uncertainties remain. This approach would support more responsive decision making and avoid non-approval or disinvestment in promising treatments because of immature or incomplete evidence.

## Suggestions to advance SACT data collection

* An expanded set of clinical outcomes becoming available from the SACT data set, going beyond overall survival and time on treatment, would be valuable if it includes more potentially decision-critical endpoints. For example, in some technology appraisals, duration of progression-free survival may be an important parameter if disease progression is associated with a large drop in health-related quality of life. We are aware that adding more data to SACT imposes costs of collection and collation, but we feel that in these cases the value of the information provided would justify its collection.
* Explore mechanisms for enabling access to individual patient-level data from SACT, possibly through partnership arrangements with the National Disease Registry Service. This could support more sophisticated analyses including subgroup analyses, adjustment for confounding, and methods such as target trial emulation. These approaches could help strengthen the credibility of real-world comparisons and increase confidence in the evidence base for decision making. The practicalities and potential added insight into the long-term outcomes of using individual-level data are being explored by a group at Newcastle University Hospitals Trust, outside of this HTA Lab project and NICE.