Australian Clinical Trials Alliance

# ACTA Submission to the Medicare Benefits Schedule Review Taskforce

November 2015

Professor Bruce Robinson
Chair, Medical Benefits Schedule Review Taskforce

9 November 2015

Dear Professor Robinson,

**Re: Submission to the Medicare Benefits Schedule Review Taskforce**

The Australian Clinical Trials Alliance (ACTA) welcomes the opportunity to make the following submission to the Medicare Benefits Schedule (MBS) Review Taskforce (the Taskforce) regarding the review of the MBS being undertaken as part of the Government's Healthier Medicare initiative.

ACTA strongly supports the objectives of the MBS Review to "*curb inefficiency by ensuring that low-value services—that is, services which provide no or negligible clinical benefit and, in some cases, might actually do harm to patients—are not funded, allowing Government investment to be directed to more effective, evidence-based services.*"

Our submission is of a macro-level nature and addresses activities 5 and 6 of the review (*Recommend changes to the rules and regulations that underpin the operation of the MBS & Embed processes for ongoing review of the MBS*).

**Our key recommendations to support the delivery of an evidence-based and value-driven MBS relate to:**

1. **Formal mechanisms for *generating* the evidence needed to address uncertainty about the benefit and/or value of new or existing services.**
2. **Reimbursement for participation in activities that generate evidence. We believe that this will not only improve outcomes but reduce wasted expenditure in the long-term.**

The MBS review is one of the most important healthcare reform initiatives to be undertaken since Medicare was first introduced. ACTA believes the opportunity to use this process to establish mechanisms that better enable the schedule itself to facilitate the generation of evidence that discriminates between low and high value services is a transformative one that will have far reaching impact on the sustainability of high-quality, universal healthcare in Australia.

We would welcome the opportunity to discuss the recommendations put forward by ACTA in further detail with the Taskforce in due course.

Professor John Zalcberg OAM
Chair, Australian Clinical Trials Alliance

# About ACTA

ACTA was established in 2014 as a national mechanism to support high-quality investigator-led clinical trials and clinical quality registries within the Australian healthcare system.

**Our mission is to promote effective and cost-effective health care in Australia through investigator-initiated clinical trials and registries that generate evidence to support decisions made by health practitioners, policymakers and consumers.**

The ACTA community currently incorporates more than 60 clinical trials networks, clinical trial coordinating centres and clinical quality registries around Australia and represents more than 10,000 senior doctors, nurses, allied health professionals and career researchers around Australia (see Appendix A).

These groups cover a broad range of disease groups and clinical disciplines and extend well into regional – and in many cases, rural – healthcare facilitates in Australia.

They are among Australia's most productive and high impact clinical researchers; responsible for establishing the effectiveness, and in some cases the harm, associated with numerous new and/or commonly used medical therapies through public-good clinical trials or identifying unwarranted variation in practice and outcomes though clinical quality registries.

A recent ACTA survey commissioned by

the National Health & Medical Research Council

demonstrated that **over the last decade**

**Australian Clinical Trials Networks** completed or initiated

over **1,000 studies** involving **more than 1 million participants**

representing **at least $1 billion in research funding** & including

**more than 100 public-good trials that have changed**

**clinical practice or policy…** [1]

[1] Australian Clinical Trials Alliance (2015) Report on the Activities & Achievements of Clinical Trials Networks in Australia 2004 – 2014 *(Report commissioned by the NHMRC and approved for publication).* Melbourne.

## An evidence-based MBS needs evidence…

It is widely acknowledged that the world-class healthcare system we enjoy today might not be affordable tomorrow. Governments around the world are in an increasingly difficult position – there is escalating demand for access to innovative new services/technologies and increasingly complex interventions from the public and the clinical community, but this must be balanced by the need to ensure effectiveness, cost-effectiveness and value for a finite health dollar.

Additionally, the burgeoning number of clinical treatments and healthcare services available today are, in part, contributing to high levels of clinical uncertainty and unwarranted variation in practice and outcomes. Applications for promising and innovative new services and technologies to be listed on the MBS are often accompanied by a limited amount of available data on the effectiveness/cost-effectiveness, particularly for new medical devices, which makes their true utility and value in real-world clinical practice difficult to reliably assess. These issues have been well documented by the Taskforce as part of the current review, which includes among its objectives identifying ways to develop a more evidence-based, value-driven MBS.

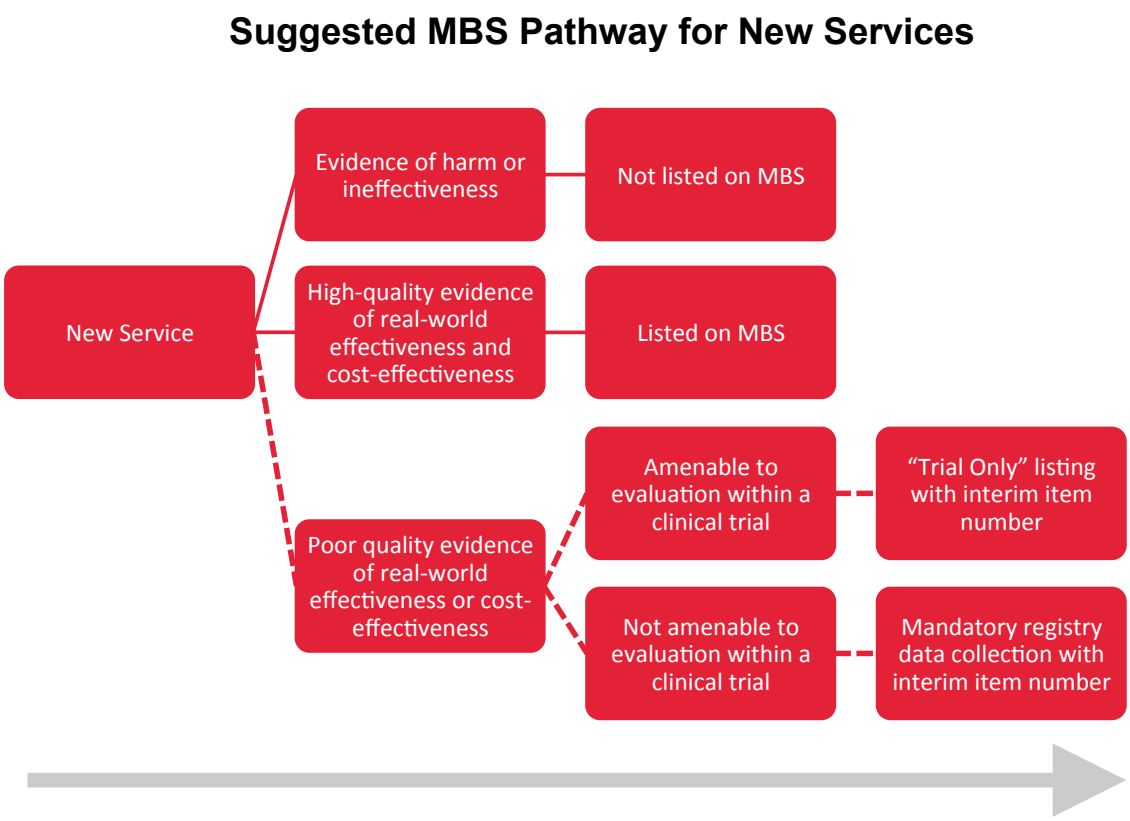
ACTA believes the success of any strategies proposed by the Taskforce will ultimately be contingent on better collection and analysis of real-world clinical and outcomes data along with better pathways for generating robust evidence of effectiveness and comparative effectiveness of new and existing services (particularly within subgroups of patients for whom clinical benefit may be uncertain).

## Formal mechanisms for generating the evidence needed to address uncertainty about the benefits and/or value of new or existing services.

The Medical Services Advisory Committee (MSAC) provides a world-class platform for Health Technology Assessment (HTA) in Australia. However, the terms of reference and policy framework within which MSAC and the MBS currently operate is largely a linear one. ACTA believes this represents a missed opportunity for the HTA assessment process (in which Government and the health sector already invests heavily) to be a dynamic, integrated and flexible platform capable of driving activities that provide reliable answers to important clinical and policy-related questions with the potential to result in major health gains for patients and/or significantly reduce waste on low or no value services funded by the public purse.

ACTA believes that the approval pathway for certain new services/technologies that are likely to be significant to patients and represent a high-cost to the MBS should be expanded to incorporate the conditional listing of the new item subject to the collection and analysis of robust clinical and patient-centred outcome data through either a randomised clinical trial or a clinical quality registry. Put simply, if the data are insufficient or questionable, the item should only be reimbursed if the patient is participating in an approved public-good trial or registry (Figure 1).

**Figure 1.**




Such a mechanism could operate in similar way to the *Coverage with Evidence Development* (CED) scheme in place in the United States whereby Medicare covers items and services on the condition that they are provided in the context of approved clinical studies or with the collection of additional clinical data to assess the utility of an item or service for use with a particular patient group [2] (a more detailed summary of the key principles and requirements of the CED scheme is provided in Appendix B).

The conceptual framework for CED is that: (1) The existing evidence has to be promising but insufficient to warrant unlimited coverage, (2) the potential population health benefit has to be significant, and (3) the research required to reduce the uncertainty would only be feasible with

[2] Medicare Coverage Document (MCD) for Guidance for the Public, Industry and CMS Staff: Coverage with Evidence Development (2014) Accessed November 2015 at https://www.cms.gov/medicare-coverage-database/details/medicare-coverage-document-details.aspx?MCDId=27

[limited] Medicare coverage.[3] Whilst these are in line with the principles of MSAC's current provision for making interim funding recommendations [4] (Box 1), the CED scheme extends beyond the initial coverage determination to provide a defined and rigorous pathway for services and technologies unproven in the real world to make it into practice sooner, but is conditional upon participation in a trial or registry capable of generating important data about the clinical effectiveness and/or value of the item in the real-world context.

**Box 1.**

**MSAC Interim Funding Criteria**

**Primary criteria for Interim Funding**

- Significant clinical need for the proposed service — is it likely to have a major effect on the morbidity and/or mortality of the disease treated?
- Some evidence of effectiveness, at least in the short term;
- Adequate evidence of safety, at least in the short to medium term; and
- Cost-effectiveness — is the new service likely to be as effective as, but less costly than, the comparator service, or more effective at a cost proportional to its increased effectiveness?

**Secondary criteria for Interim Funding**

- Potential for further studies to reduce uncertainty and to obtain relevant answers.
- Cost–benefit of conducting the study in relation to the value of the answer (in terms of likely utilisation under Medicare).
- Lack of information elsewhere (eg from overseas studies).
- Lack of alternative funding sources (eg commercial sponsors).

Similarly, where existing items are identified as being of potentially low value during the current MBS review process (and beyond), the opportunity exists for the schedule itself to be used to generate evidence to discriminate between low and high value services. As with the process for new items identified above, a condition of billing for existing items identified as being of potentially low value should be submission of limited but key data to identify the characteristics of patients receiving the service and the patient-centred clinical outcomes for that individual patient.

For potentially low value items, particularly those not amenable to evaluation within a randomised controlled trial, these data can be analysed to draw inference about the true value of the service, and in particular, identify patient characteristics that are associated with no or low value to guide future policy.

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[3] Tunis, SR & Pearson SD (2006) "Coverage Options For Promising Technologies: Medicare's 'Coverage With Evidence Development." Health Affairs, 25:5 1218-1230

[4] Proposal for Changes to the Medical Services Advisory Committee (MSAC) Processes for Applications for Public Funding: Discussion Paper (2009). Accessed November 2015 at http://www.msac.gov.au/internet/msac/publishing.nsf/content/B6E5CA7CC1CA236DCA257ABC00182ABD/$File/MSAC%20Discussion%20Paper.pdf

**A unique opportunity to develop innovative funding models for public-good clinical trials and registries.**

- One of the consequential outcomes of making an item available only within the context of a randomised controlled trial is that the MBS will only be billed for half of the patients that would have otherwise received the unproven new service or technology. This provides a unique opportunity to reinvest a proportion of the direct cost savings associated with this approach to support the cost of the trial.
- Another consequence of providing reimbursement for an unproven new service /technology or an existing item of potentially low value within a clinical trial is the potential for costs savings associated with the rigor of trial protocols which act to control the frequency of interventions such as pathology tests and diagnostic imaging. ACTA suggest that this is another mechanism through which Government could seek to identify savings to offset the cost of conducting large public-good trials and establishing and maintaining clinical quality registries.
- Using the MBS as a much stronger driver for the development of large public-good trials and registries is likely to encourage more partnership between governments, clinical communities, industry and the not-for-profit sector to fund these activities, such as the consortium of public/private partners that formed to establish the Bariatric Surgery Registry.

**Leveraging existing HTA capacity.**

ACTA believes that a system similar to the CED scheme could be readily implemented in Australia where we have a number of well-established and highly successful clinical trials networks upon which to model the successful conduct of large public-good trials as well as world-class expertise in the development and operation of clinical quality registries.

**Key Recommendations:**

- **The Taskforce should consider the adoption of a system similar to the Coverage with Evidence Development mechanism in the United States to provide "Trial Only" or "Registry Only" listing for new or existing services where the available evidence is insufficient to reliably determine clinical benefit and/or comparative value in the real-world context.**
- **A review of potential reforms to the MBS aimed at facilitating better generation of real-world evidence to improve outcomes and deliver value gains should consider ways to use savings generated through public-good trials and registries as a means of funding these activities.**

# Reimbursement for participation in activities that generate evidence.

It has been articulated many times that successful healthcare reform in Australia will require embedding processes to routinely measure and report health outcomes and robustly evaluate the comparative clinical benefits of competing treatment options into the core business of healthcare delivery. In reality, achieving this is a massive task that requires a complex strategy incorporating multiple levers impacting at all levels of the healthcare system to drive a major cultural shift towards excellence through innovation *within* the system.

Compared to countries like the United Kingdom, United States and Sweden, Australia has made far less progress towards integrating research into routine healthcare delivery and the outcomes of the MBS review are likely to be another stark reminder that our healthcare system lacks the embedded culture, processes and infrastructure to support routine participation in high-quality clinical/health services research and the measurement of clinical outcomes.

ACTA believes that this review offers a critical opportunity to take a bold and visionary step towards truly embedding research into clinical practice by initiating work to pilot the introduction of an MBS item number to reimburse clinicians for activities that generate evidence, such as the enrolment of a patient into an approved public-good clinical trial or submission of data to an approved clinical quality registry.

Investigating the possibility of changes to the MBS to facilitate greater clinician and patient involvement in the conduct of post-implementation studies was a longer-term direction recommended by the Review of Health Technology Assessment in Australia published in 2009.[5] Notwithstanding the obvious complexities of designing and modelling an appropriate reimbursement policy, we believe that such a scheme is long overdue and would not only lead to better generation of new knowledge and evidence by boosting participation in important pubic-good trials and registries, but ultimately save money and directly improve quality of care.

**Key Recommendation:**

**The Taskforce should investigate piloting the introduction of a specific MBS item number for activities that generate evidence such as enrolling a patient in an approved public-good clinical trial or clinical quality registry.**

[5] Commonwealth of Australia (2009) Review of Health Technology Assessment in Australia. Canberra. Accessed November 2015 at http://www.health.gov.au/internet/main/publishing.nsf/Content/AF68234CE9EB8A78CA257BF00018CBEB/$File/hta-review-report.pdf

# Appendix A: Members of the ACTA Community

1. Australasian Child and Adolescent Obesity Research Network (ACAORN)
2. Australasian College for Emergency Medicine Clinical Trials Group (ACEM Clinical Trials Group)
3. Australasian Consortium of Centres for Clinical Cognitive Research (AC4R)
4. Australasian Gastro-Intestinal Trials Group (AGITG)
5. Australasian Kidney Trials Network (AKTN)
6. Australasian Lung Cancer Trials Group (ALTG)
7. Australasian Radiopharmaceutical Trials Network (ARTnet)
8. Australasian Sarcoma Study Group (ASSG)
9. Australasian Sleep Trials Network (ASTN)
10. Australasian Society for Infectious Diseases Clinical Research Network (ASID CRN)
11. Australasian Stroke Trials Network (ASTN)
12. Australia & New Zealand Breast Cancer Trials Group (ANZBCTG)
13. Australia & New Zealand Neonatal Network (ANZNN)
14. Australia & New Zealand Society of Cardiac & Thoracic Surgeons (ANZSCTS) National Cardiac Surgery Database
15. Australia New Zealand Gynaecological Oncology Group (ANZGOG)
16. Australian & New Zealand Children's Haematology/Oncology Group (ANZCHOG)
17. Australian & New Zealand College of Anaesthetists Clinical Trials Network (ANZCA CTN)
18. Australian & New Zealand Intensive Care Society Centre for Outcomes & Resource Evaluation (ANZICS CORE)
19. Australian & New Zealand Intensive Care Society Clinical Trials Group (ANZICS CTG)
20. Australian & New Zealand Melanoma Trials Group (ANZMTG)
21. Australian & New Zealand Musculoskeletal Clinical Trials Group (ANZMUSC)
22. Australian & New Zealand Urogenital & Prostate Cancer Trials Group (ANZUP)
23. Australian Epilepsy Clinical Trials Network (AECTN)
24. Australian Motor Neuron Disease Registry (AMNDR)
25. Australian Neuromuscular Network (ANN)
26. Australian Orthopaedic Association National Joint Replacement Register (AOANJRR)
27. Australian Paediatric Research Network (APRN)
28. Australian Primary Care Research Network (APCReN)
29. Australian Research Centre for Health of Women & Babies, Robinson Institute.
30. Bariatric Surgery Registry (BSR)
31. Bi-national Colorectal Cancer Audit (BCCA)
32. Burns Service of Western Australia
33. Centre for Anaesthesia & Cognitive Function
34. Centre for Biostatistics & Clinical Trials (BaCT)
35. Cooperative Trials Group for Neuro-Oncology (COGNO)
36. Multiple Sclerosis Research Australia Clinical Trials Network (MSRACTN)
37. Neuroscience Trials Australia (NTA)
38. NHMRC Clinical Trials Centre (NHMRC CTC)
39. NSW Better Treatments 4 Kids (BT4K)
40. Orygen Youth Health Research Centre
41. Paediatric Research in Emergency Departments International Collaborative (PREDICT)
42. Paediatric Trials Network Australia (PTNA)
43. Palliative Care Clinical Studies Collaborative (PaCCSC)
44. Perinatal Society of Australia & New Zealand IMPACT Collaboration
45. Primary Care Collaborative Cancer Clinical Trials Group (PC4)
46. Prostate Cancer Clinical Quality Registry
47. Psycho-oncology Co-operative Research Group (PoCoG)
48. Queensland Centre for Mental Health Research
49. Queensland Clinical Trials & Biostatistics Centre
50. School of Public Health & Preventative Medicine, Monash University
51. South Australian Health & Medical Research Institute (SAHMRI)
52. Spinal Cord Injury Network (SCIN)
53. The ASPREE Study Group
54. The George Institute for Global Health
55. Therapeutic and Vaccine Research Program (TVRP), The Kirby Institute for Infection and immunity in Society
56. Trans-Tasman Radiation Oncology Group (TROG)
57. Transfusion Research Outcomes Collaborative (TORC)
58. Type 1 Diabetes Clinical Research Network (T1DCRN)
59. Victorian Ambulance Cardiac Arrest Registry
60. Victorian Cardiac Outcomes Registry (VCOR)
61. Victorian Cervical Cytology Registry (VCCR)
62. Victorian State Trauma Outcomes and Monitoring Registry (VSTORM)

# Appendix B: United States Centres for Medicare & Medicaid Services (CMS) Coverage with Evidence Development (CED)

*Extracted from "Medicare Coverage Document (MCD) for Guidance for the Public, Industry and CMS Staff: Coverage with Evidence Development" available at https://www.cms.gov/medicare-coverage-database/details/medicare-coverage-document-details.aspx?MCDId=27*

## Principles governing the application of CED

- CED will occur within the coverage determination process, which is transparent and open to public comment.
- CED will not be used when less restricted coverage is justified by the available evidence.
- CED will generally expand access to medical technologies for beneficiaries.
- CED will lead to the production of evidence complementary to existing medical evidence.
- CED will not duplicate or replace the FDA's authority in assuring the safety, efficacy, and security of drugs, biological products, and devices.
- CED will not assume the NIH's role in fostering, managing, or prioritizing clinical trials.
- CED will be consistent with federal laws, regulations, and patient protections.

## Requirements for CED

We would not anticipate approving a study [under CED] that does not meet these requirements:

a) The principal purpose of the study is to test whether the item or service meaningfully improves health outcomes of affected beneficiaries who are represented by the enrolled subjects.

b) The rationale for the study is well supported by available scientific and medical evidence.

c) The study results are not anticipated to unjustifiably duplicate existing knowledge.

d) The study design is methodologically appropriate and the anticipated number of enrolled subjects is sufficient to answer the research question(s) being asked in the National Coverage Determination.

e) The study is sponsored by an organization or individual capable of completing it successfully.

f) The research study is in compliance with all applicable Federal regulations concerning the protection of human subjects found in the Code of Federal Regulations (CFR) at 45 CFR Part 46. If a study is regulated by the Food and Drug Administration (FDA), it is also in compliance with 21 CFR Parts 50 and 56. In addition, to further enhance the protection of human subjects in studies conducted under CED, the study must provide and obtain meaningful informed consent from patients regarding the risks associated with the study items and/or services, and the use and eventual disposition of the collected data.

g) All aspects of the study are conducted according to appropriate standards of scientific integrity.

h) The study has a written protocol that clearly demonstrates adherence to the standards listed here as Medicare requirements.

i) The study is not designed to exclusively test toxicity or disease pathophysiology in healthy individuals. Such studies may meet this requirement only if the disease or condition being studied is life threatening as defined in 21 CFR §312.81(a) and the patient has no other viable treatment options.

j) The clinical research studies and registries are registered on the www.ClinicalTrials.gov website by the principal sponsor/investigator prior to the enrollment of the first study subject. Registries are also registered in the Agency for Healthcare Quality (AHRQ) Registry of Patient Registries (RoPR).

k) The research study protocol specifies the method and timing of public release of all prespecified outcomes to be measured including release of outcomes if outcomes are negative or study is terminated early. The results must be made public within 12 months of the study's primary completion date, which is the date the final subject had final data collection for the primary endpoint, even if the trial does not achieve its primary aim. The results must include number started/completed, summary results for primary and secondary outcome measures, statistical analyses, and adverse events. Final results must be reported in a publicly accessibly manner; either in a peer-reviewed scientific journal (in print or on-line), in an on-line publicly accessible registry dedicated to the dissemination of clinical trial information such as ClinicalTrials.gov, or in journals willing to publish in abbreviated format (e.g., for studies with negative or incomplete results).

l) The study protocol must explicitly discuss beneficiary subpopulations affected by the item or service under investigation, particularly traditionally underrepresented groups in clinical studies, how the inclusion and exclusion criteria effect enrollment of these populations, and a plan for the retention and reporting of said populations in the trial. If the inclusion and exclusion criteria are expected to have a negative effect on the recruitment or retention of underrepresented populations, the protocol must discuss why these criteria are necessary.

m) The study protocol explicitly discusses how the results are or are not expected to be generalizable to affected beneficiary subpopulations. Separate discussions in the protocol may be necessary for populations eligible for Medicare due to age, disability or Medicaid eligibility.