Australian Government
Department of Health
Therapeutic Goods Administration

Updates from the Prescription Medicine Authorisation Branch

Dr Nitin Bagul
Prescription Medicines Authorisation Branch
Australian Government Department of Health, TGA
ARCS Annual Conference 2022

24 May 2022

TGA Health Safety Regulation

Session overview

- Registration applications and performance
- International collaborations
- Real World Evidence
- Repurposing
- Other activities

Performance highlights

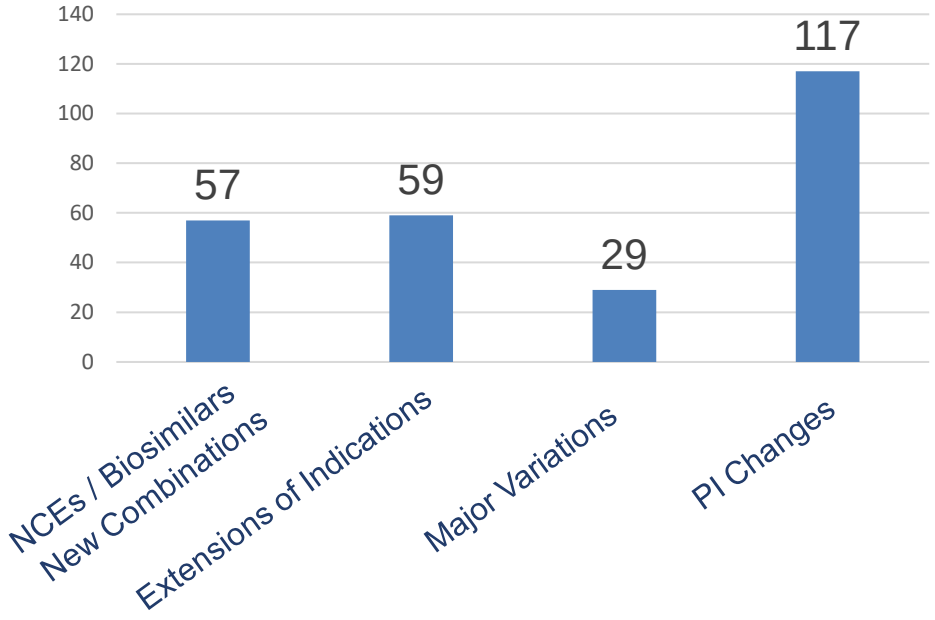
- Maintaining performance standards during COVID-19
- Rapid approval of COVID vaccine and treatment applications
- Supporting the Department's COVID response
- Increasing international collaborations

Registration applications and performance

Prescription Medicines – 2021

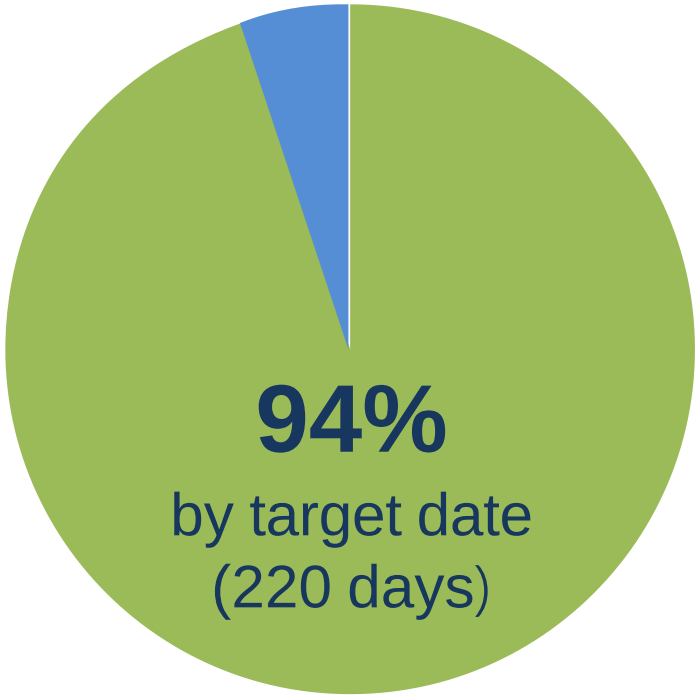
Category 1 and COR Registrations

High volume of major application types



Category 1 approval times

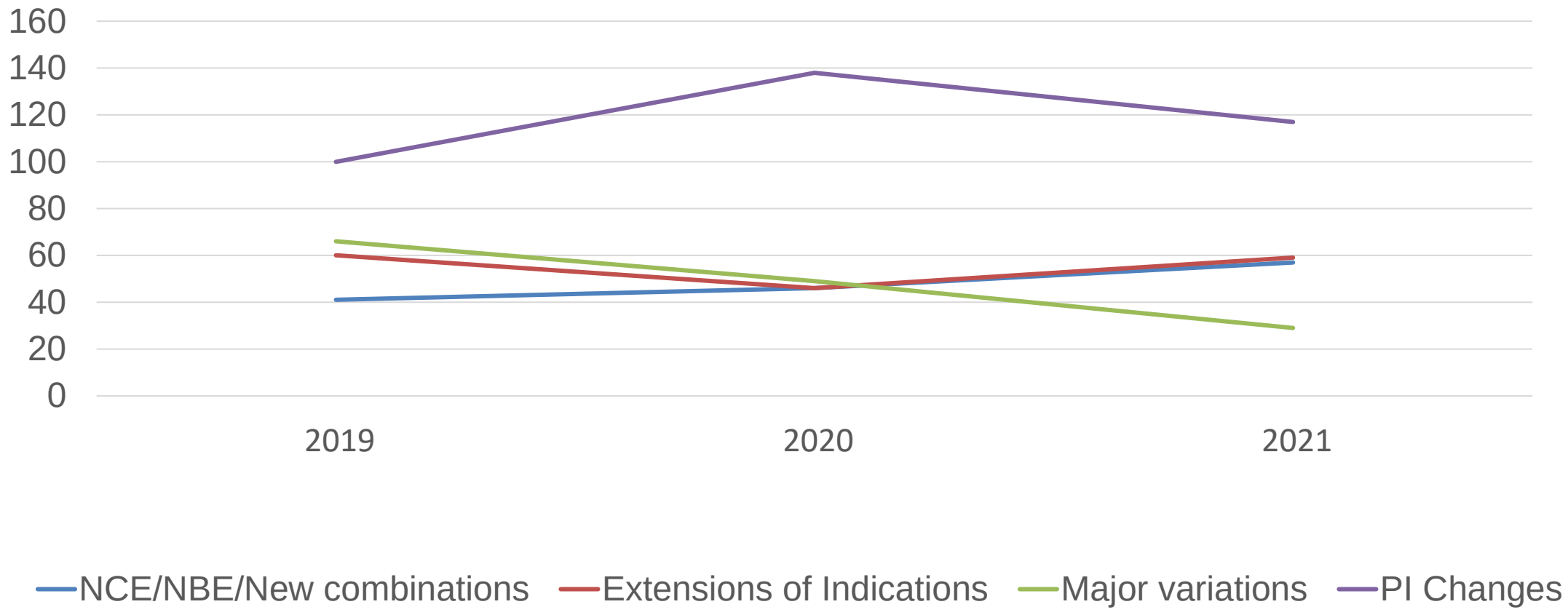
Timeframes are being met



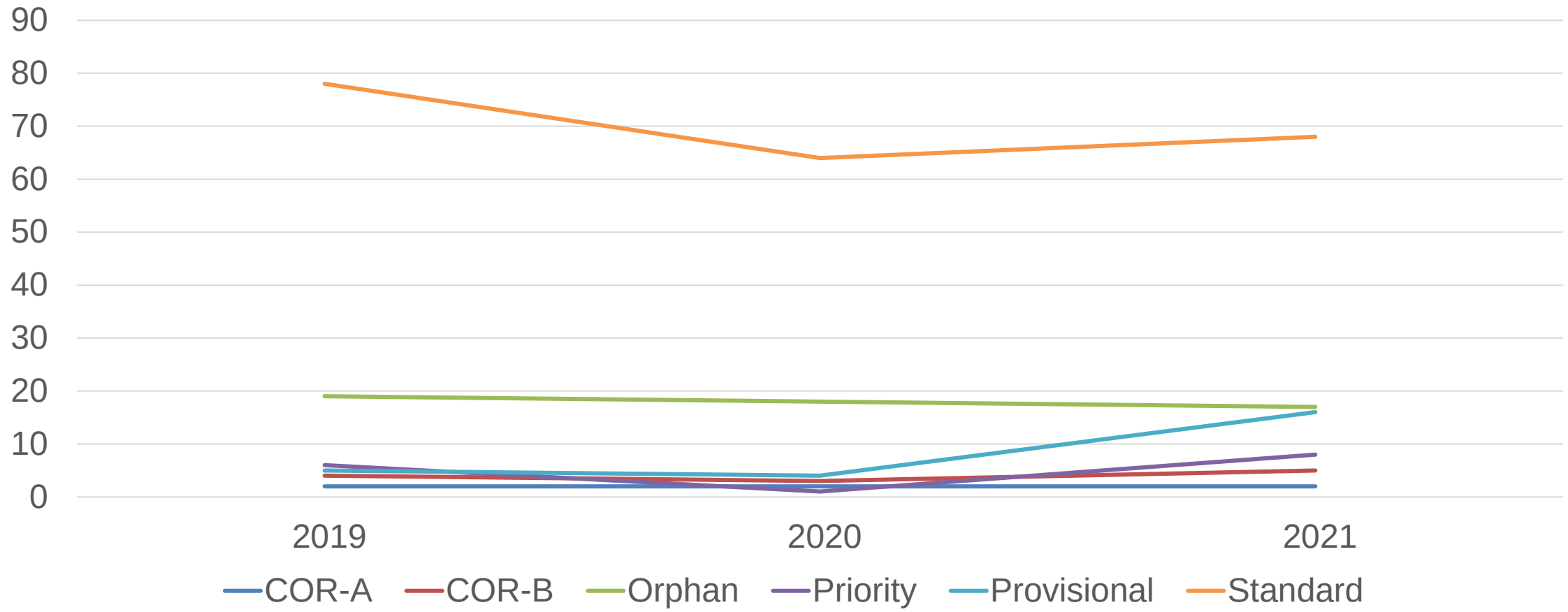
100%

before due date
(255 days)

Major application registrations over past 3 years

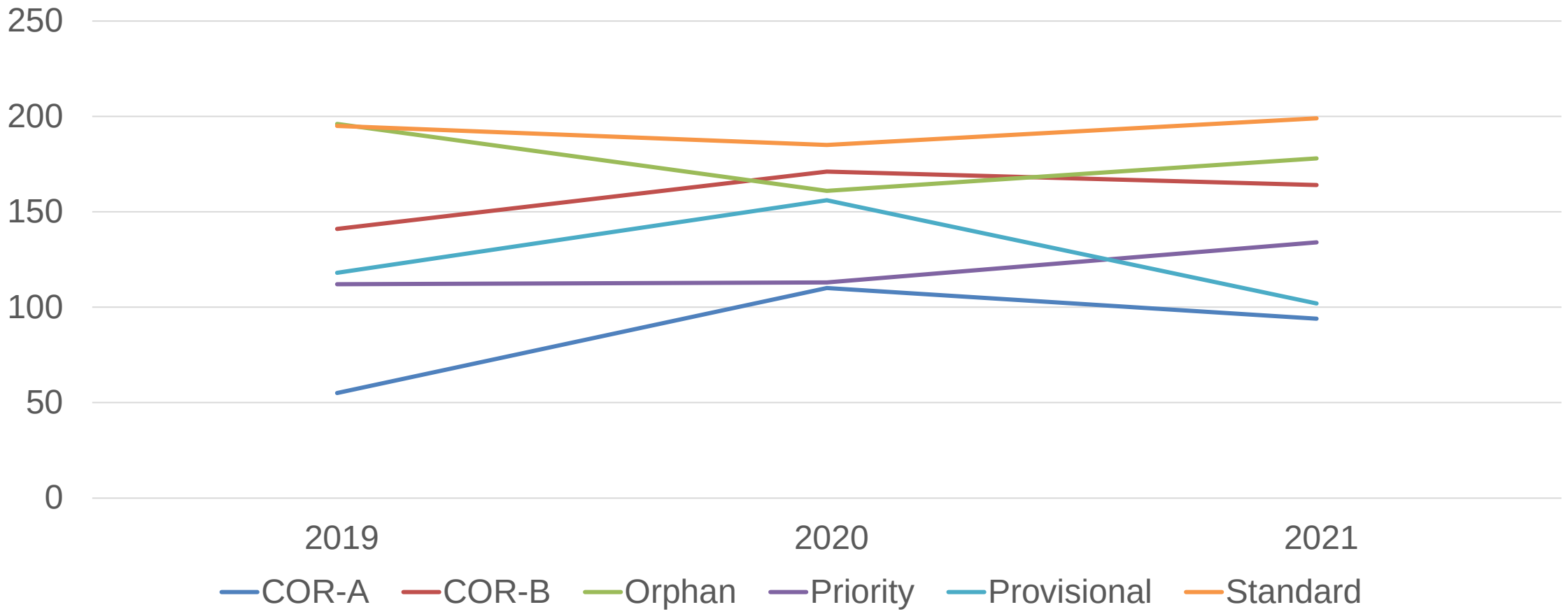


New indication approval quantities

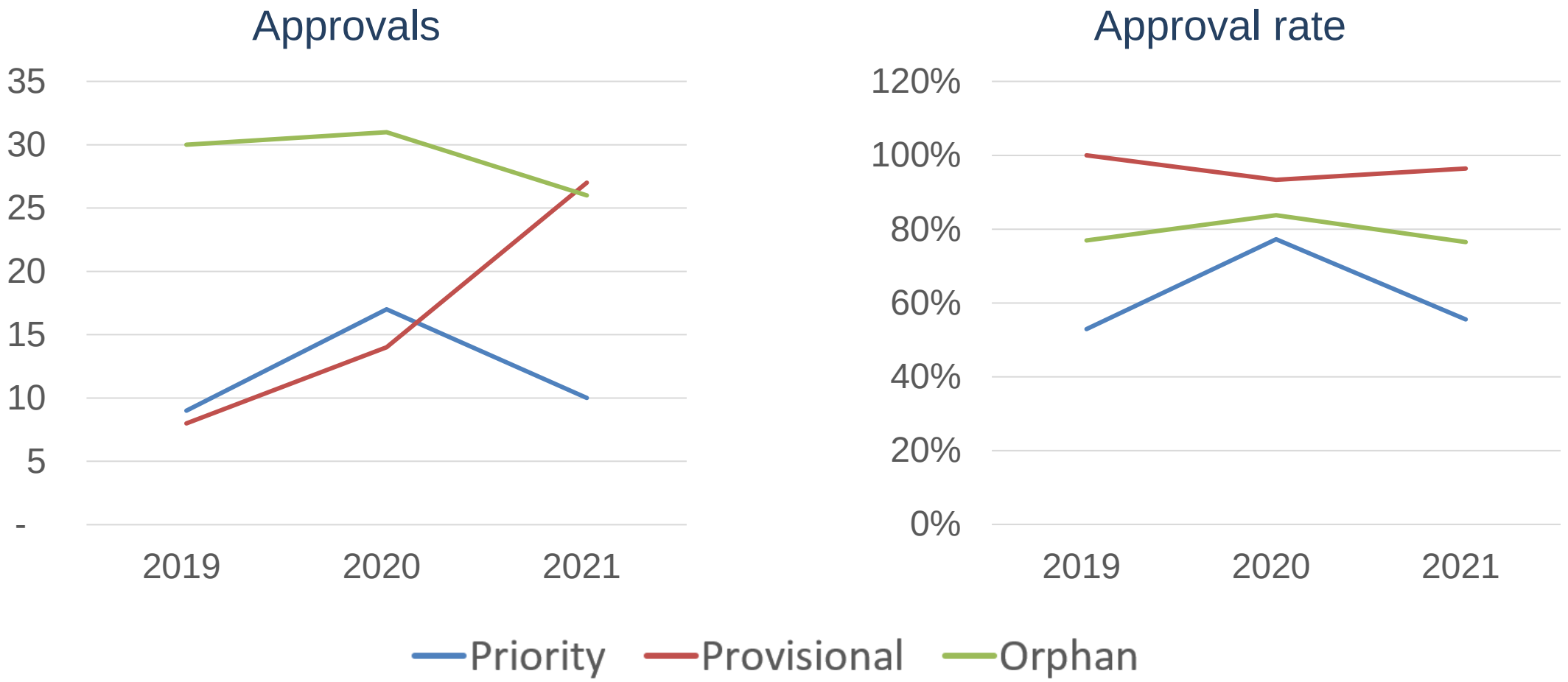


New indication approval timeframes (over COVID)

Mean approval times for submissions by pathway



Determinations & designations



Trends and observations

- New technologies
  - Increased biologics
  - Increased medicine/device interfaces, biologic/medicines
- Regulating the boundaries - Increased medicine/device interfaces
- Increased international engagement/work-sharing

International collaborations

Highlights

- Unprecedented international collaboration to provide timely access to COVID treatments and vaccines
- Strong growth in established collaborative review pathways (Access work-sharing and Project Orbis)
- Launch of Access biosimilars work-sharing

ICMRA
International Coalition of Medicines Regulatory Authorities

ACCESS CONSORTIUM
AUSTRALIA
CANADA
SINGAPORE
SWITZERLAND
UNITED KINGDOM

PROJECT ORBIS

World Health Organization

Enhanced international collaboration for COVID therapeutic goods

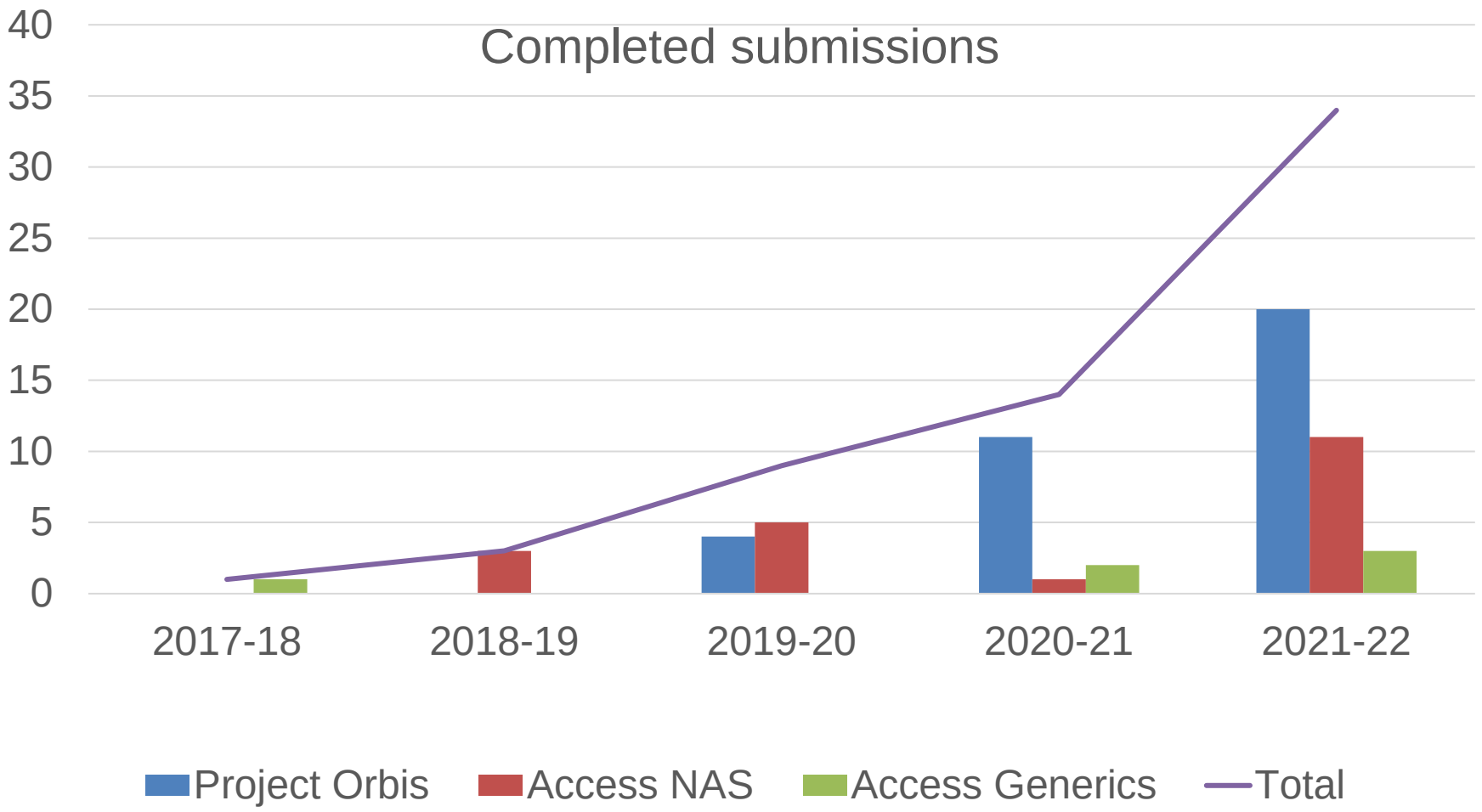
- **Regular updates on clinical trials and observational studies**
  - Early efficacy and safety signals
  - Especially important for a medium sized regulator in a country with lower COVID-19 caseload
- **Sharing (and addressing?) of concerns**
  - Clinical trials that are not sufficiently powered to gather evidence
  - Need for master clinical trial protocols and agreed endpoints, compare multiple treatments
  - Which populations should promising vaccines be made available to
  - Alignment of post-approval requirements for medicines and vaccines
- **Sharing of information on:**
  - regulatory flexibilities, policies, pipelines, submissions and evaluations
- **Better collaboration** – better approach than independent duplication of effort!
  - But will COVID-19 catalyse greater ongoing collaboration? More joint product evaluations?

Achievements so far…

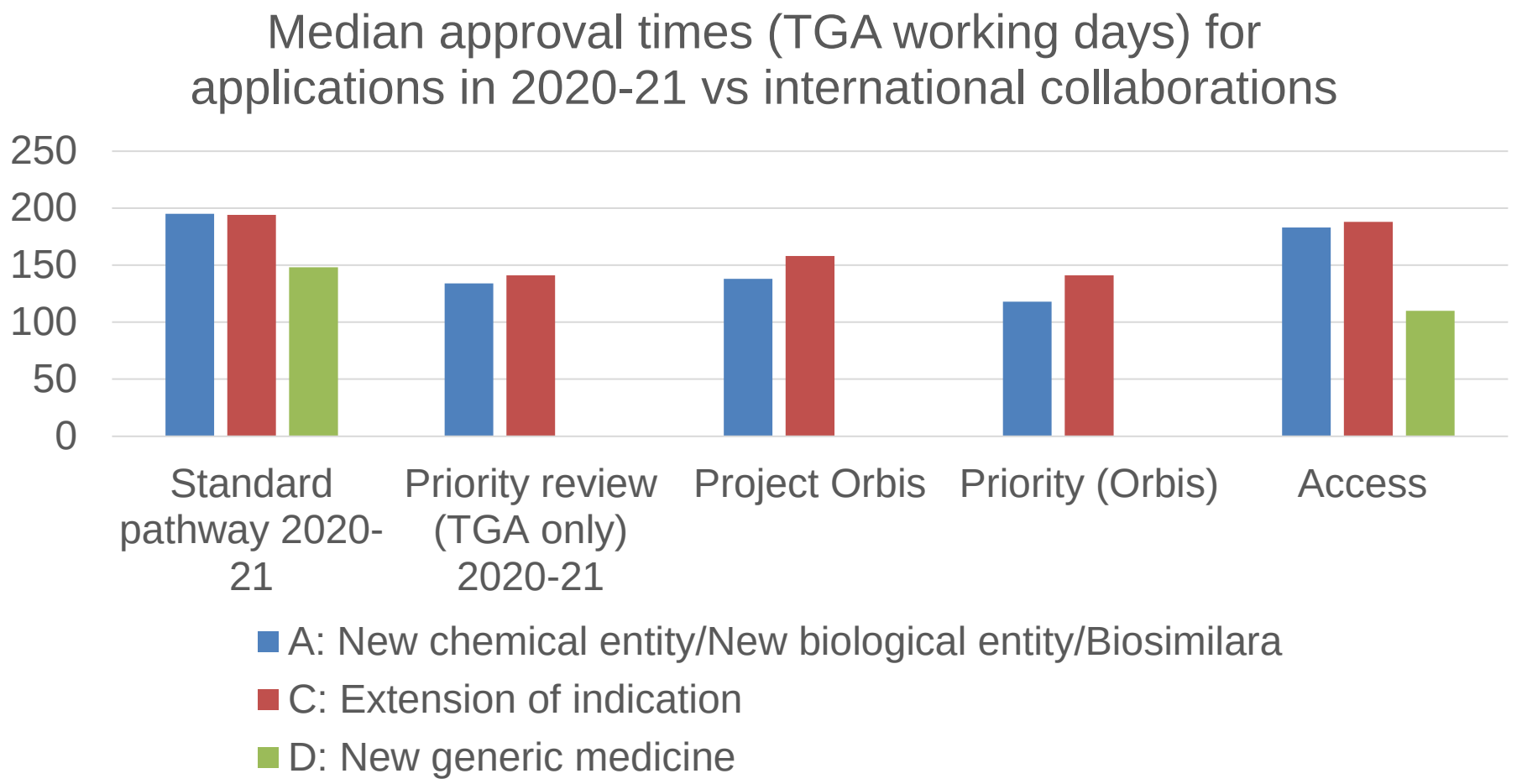
Number of completed international collaboration applications by year

	Year						
Collaboration Type	2017-18	2018-19	2019-20	2020-21	2021-22	Total	On hand
Project Orbis	0	0	4	11	20*	35	25
Access New Active Substance work-sharing	0	3	5	1	11*	20	6
Access generic medicines work-sharing	1	0	0	2	3*	6	3

International collaborations – performance highlights



Approval time comparison



International collaborations - reminders

- **Advance Notice**: Early interactions with regulators to assess whether collaborative review is a feasible
- **Australian specific requirements:** still need to be met (e.g., module 3 requirements)
- **Choose your pathway early:** Applicants need time to submit priority and provisional designation applications
- **Planning reimbursement applications**: steps, and time required between, in the decision phase vary depending on the complexity of the specific submission

More information

- Access NASWSI Operational Procedures published September 2021
  - https://www.tga.gov.au/access-consortium-new-active-substance-nas-work-sharing-initiative

- TGA guidance on Project Orbis published in February 2022
  - https://www.tga.gov.au/project-orbis

- Information about completed Access and Orbis applications included decision summaries and AusPARs

Coming soon

- Information about completed Access and Orbis applications to be included on ‘new registrations’ pages

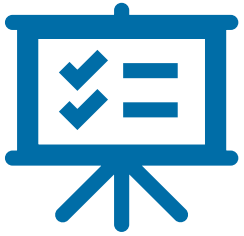
Real World Evidence

Real World Evidence

- Consultation in 2021 revealed industry and consumers are unclear on how the TGA uses RWE
- Increasing availability of RWE
- Opportunities for improved treatments and repurposing of medicines
- Clinical trial evidence versus RWE in applications

What is the TGA doing?

Opening Dialogue



Setting definitions and guidelines

Increasing our transparency on RWE

Increase communication and understanding of RWE amongst internal and external stakeholders

TGA response 2021 – 2022

- Establish a central point for RWE information on TGA website
- Develop Australian RWE definition
- Collaborate with overseas regulators
- Consult on the development and adoption of guidance documents
- Provide guidance around use of RWE for pre-market evaluations
- Communicate when RWE has been used to make a regulatory decision
- Support RWE use in Orphan, Provisional and Repurposing of medicines

Repurposing

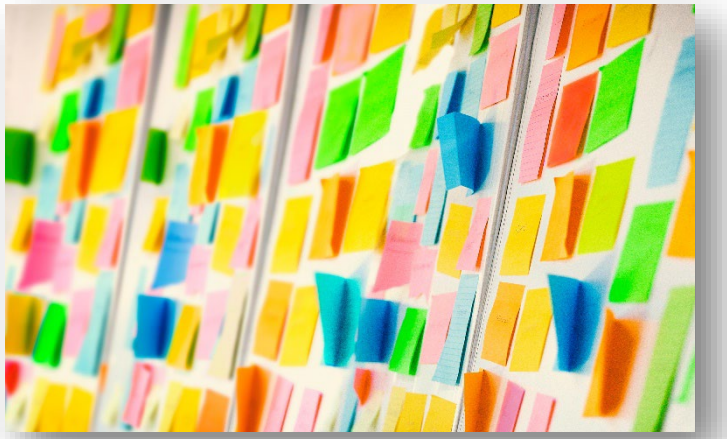
Government decisions to take

The Government has agreed for the Department to work toward a policy that:

- Pro-actively identifies suitable applications for evaluation of medicines for repurposing
- Provides support to the development of regulatory and reimbursement applications including collation of clinical evidence, literature reviews, and sourcing reviews from CORs and HTA
- Provides priority review for evaluations
- Considers application and evaluation fees
- Considers regulatory exclusivity for a limited period for sponsors for repurposed indications
- Abridges applications focussing on clinical efficacy, effectiveness and safety to enable a repurposed off-patent indication
- Allowing groups of willing sponsors to jointly extend indications within a highly coordinated TGA and PBAC evaluation process
- Where there is no current sponsor interested in submitting an off-patent medicine application, the seeking of expressions of interest for sponsorship

Work to date

- Initial conceptual work
- 2021 consultation
  - Public feedback and ideation
  - May workshop
  - July roundtables
- Problem and benefit definition
- Engagement with government
- Process development including potential criteria and flow
- 2022 consultation
  - Public feedback and ideation
  - To come: small targeted workshops



Key points from 2022 consultation responses

- Repurposing medicines and reducing regulatory burden welcomed
- All stakeholders want early involvement to ensure patient voice, safety, efficacy, commercial viability and regulatory factors considered at outset
- Clear guidance and criteria to be set by the Department, including RWE usage
- Strong sentiment for ‘patient voice’, unmet need, patient care and QoL was raised
- For collaborative submissions, non-competitive environment and engagement towards a common goal
- Concerns regarding extension of data or market exclusivity and market monopoly
- Transparency needed around candidate identification and selection process

A few reminders

A short plug

- Dr Andrew Leaver will present more about real world evidence in the session this afternoon @ 4.30pm
  - **B26 – The evolving role of real world evidence in supporting access to medicines**

- Dr Mohit Khera presented more about TGA COVID-19 learnings in the session yesterday @ 1.30pm
  - **A11. Regulatory aspects of vaccine development**

Transition to eCTD for prescription medicines

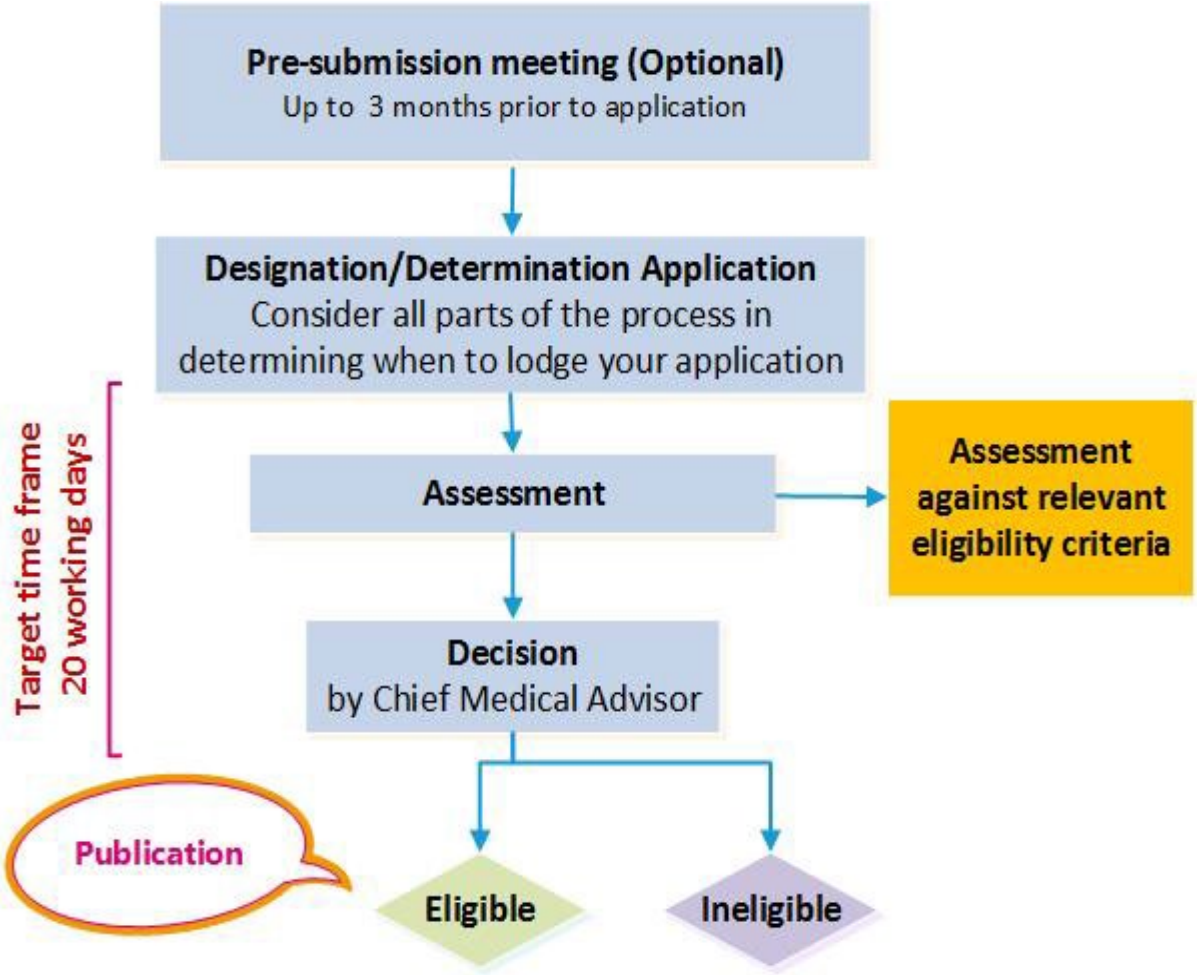
- **1 November 2021 – eCTD-Only Stage 1**
  - New Chemical Entity Medicine (Type A)
  - New Biological Entity Medicine (Type A)
  - New Biosimilar Medicine (Type A)
  - New Combination Medicine (Type B)
- **1 June 2022 – eCTD-Only Stage 2**
  - Extension of Indications Medicine (Type C)
  - Major Variation Medicine (Type F)
  - New Generic Product (Type D)
- **1 November 2022 – eCTD-Only Stage 3**
  - All remaining prescription medicine data including master files

Transition to eCTD for prescription medicines

- **Once eCTD is mandated for your stage we expect the following to be provided as eCTD sequences:**
  - All category 1 applications
  - All minor variation applications
  - Other prescription medicine applications (Type O and Type E)
  - PSURs
  - RMP updates
  - CPDs
  - Section 14 exemptions
  - Prescription medicine master files

- **Other data can be provided in the eCTD format including:**
  - Designation and Determination applications
  - Clinical Trial Applications (CTA)

Determination/designation: Process



- Only medicines likely to provide the most benefit are eligible
- A designation/determination must be in force to access pathways and/or fee waiver
- Designation/determination in force for 6 months
- 6 months extension if registration application not submitted (1-month prior expiry not for priority)
- Sponsor may re-apply for orphan designation once lapsed, but criteria ***must*** be met (e.g., show benefit against self)
- Orphan designation applicable to all 5 pathways (standard, priority, provisional, COR A & B)
- Consistent & transparent process

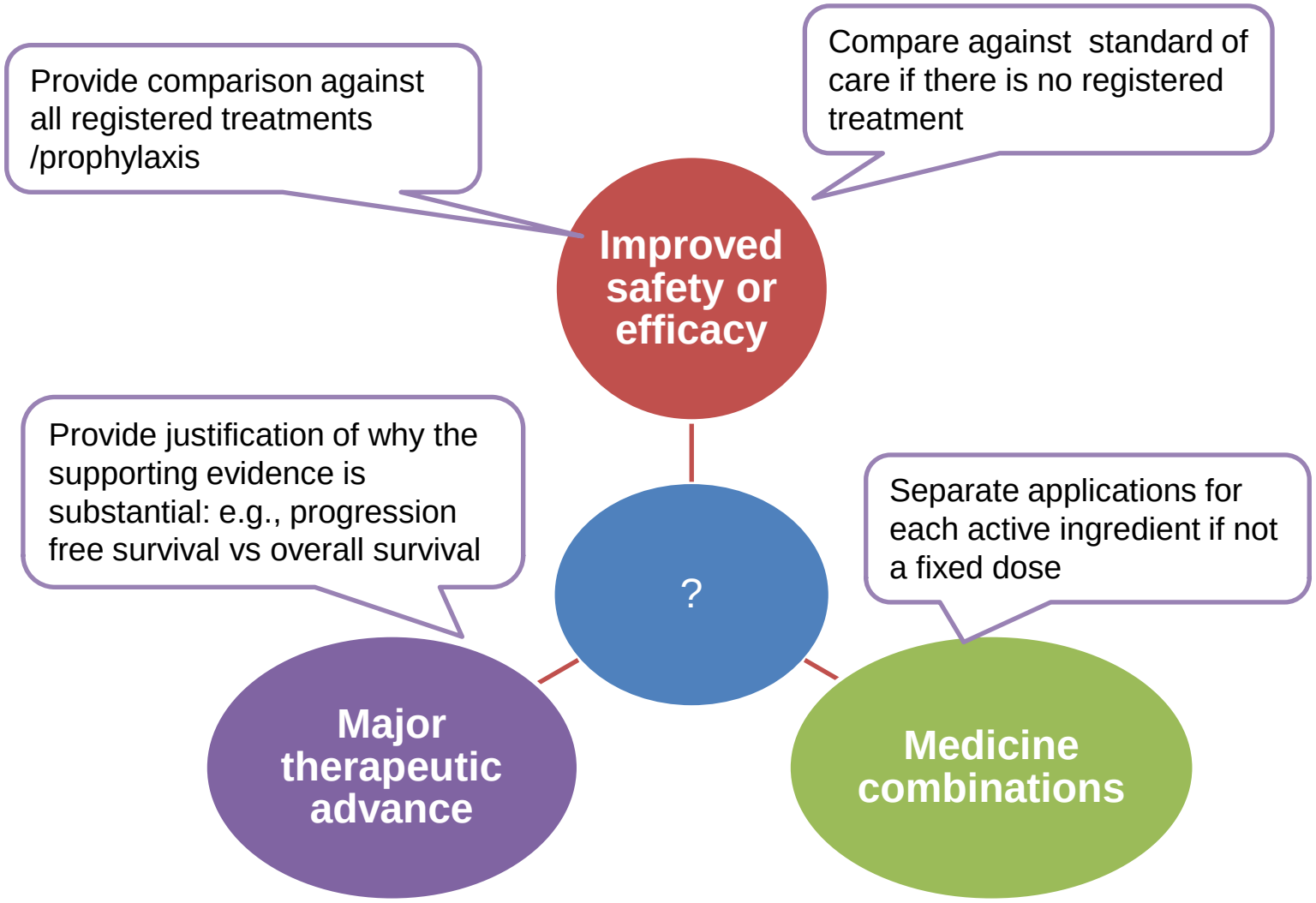
Designation/determination: Reminders

Main rejection reasons:

- Comparison against registered therapeutic goods – *the criteria are assessed at the time the decision is made*
- Major therapeutic advance

Orphan drug considerations:

- Prevalence: calculated differently for diagnosis, prevention or treatment
- Subgrouping:
  - compare against standard of care in the absence of a registered treatment
  - line of therapy or disease stage are not a valid subgroup



Questions?

Australian Government

Department of Health

Therapeutic Goods Administration