Industry Research and Development (clinical trials, phase 0, I, II, III, pre-market pilot stage, pre-market pivotal stage, for an unapproved therapeutic good) Determination 2022

I, **Andrew Stevens**, Acting Chairperson, Industry Innovation and Science (the Board), in accordance with subsection 31D of the *Industry Research and Development Act 1986,* make the following determination.

Dated: 30 March 2022

Andrew Stevens

Acting Chair

Industry Innovation and Science Australia

Contents

Part 1 – Preliminary

1 Name

This instrument is the *Industry Research and Development (clinical trials, phase 0, I, II, III, pre-market pilot stage, pre-market pivotal stage, for an unapproved therapeutic good) Determination 2022*.

2 Commencement

This instrument is taken to have commenced on the day after it is registered on the Federal Register of Legislation.

3 Authority

This instrument is made under subsection 31D(1) of the *Industry Research and Development Act 1986*.

4 Definitions

Note: A number of expressions used in this instrument are defined in Division 355 of the *Income Tax Assessment Act 1997*, including the following:
(a) core R&D activities; and
(b) R&D entity.

In this instrument:

biosimilar *medicine* means a version of an already-registered biological medicine. These medicines may be referred to as similar biological medicinal products, similar biotherapeutic products, subsequent entry products, bioequivalents, or follow-on products. Both the biosimilar and its reference medicine will have the following similar characteristics (demonstrated using comprehensive comparability studies): physicochemical, biological, immunological, efficacy and safety.

generic product means a medicine that, in comparison to a registered medicine or a medicine that has been registered but is no longer a registered medicine (the comparison medicine):
(a) has the same quantitative composition of therapeutically active substances, being substances of similar quality to those used in the comparison medicine; and
(b) has the same pharmaceutical form; and
(c) is bioequivalent; and
(d) has the same safety and efficacy properties.

phase 0 clinical trial *(human pharmacology (micro-dosing))* means a clinical trial that involves dosing a limited number of humans with a limited range of doses of a medicine or biological for a limited period of time with the objective of:
(a) assessing pharmacokinetics; and
(b) gathering preliminary data on pharmacokinetics and bioavailability to determine if the drug behaves as expected from preclinical studies 'micro-dosing' studies.

phase I clinical trial *(human pharmacology)* means a clinical trial that involves the first administration to humans of a medicine or biological with the objective of assessing its safety and tolerance. In includes defining or describing pharmacokinetics

and pharmacodynamics; determining dosing; exploring drug metabolism and drug interactions; and identifying preferred routes of administration.

(a) Phase Ia: a clinical trial that involves a single ascending dose.
(b) Phase Ib: a clinical trails that involves a multiple ascending dose.

phase II clinical trial *(therapeutic exploratory)* means a clinical trial undertaken in a larger group of human patients with the objective of evaluating the efficacy and safety of a medicine or biological.

(c) Phase IIa: a clinical trialtrials to demonstrate clinical efficacy or biological activity through pilot studies and to explore therapeutic dose range.
(d) Phase IIb: a clinical trial to determine optimum therapeutic dose and regimen (with efficacy as primary endpoint); and to resolve uncertainties regarding the design and conduct of subsequent trials.

phase III clinical trial *(therapeutic confirmatory)* means a clinical trial that involves a large group of patients and aims to evaluate the safety, efficacy or effectiveness of a medicine or biological.

(a) Phase IIIa: a clinical trial to determine the therapeutic effect in patient populations for which the drug is eventually intended. It provides a definitive assessment of risk-benefit balance (to support drug registration or change in clinical practice).
(b) Phase IIIb: a clinical trial to increase patient exposure and support marketing claims or publication.

phase IV clinical trial *(therapeutic use)* means a clinical trial of a medicine or biological that includes: post-marketing surveillance or resolution of treatment uncertainties; monitoring safety in real-world populations; refining knowledge of the risk-benefit balance, detecting rare or long-term adverse effects, and drug interactions; pharmacoeconomics to gather data in support of the use; comparative effectiveness and community-based research (sometimes described as Phase V trials); and trial combinations with existing products.

post-market stage clinical trial means a confirmatory investigation of a medical device to establish performance and safety; or observational investigations or surveillance to gain a better understanding of device safety, long-term outcomes, and health economics.

pre-market pilot stage clinical trial means a clinical trial of a medical device that involves a small group of human patients with the objective of being an exploratory investigation to determine preliminary safety and performance information to plan design modifications or provide support for a future pivotal study. It includes first in human and feasibility studies or proof of concept.

pre-market pivotal stage clinical trial means a clinical trial of a medical device that involves a confirmatory investigation to evaluate performance and safety for a specified intended use to satisfy pre-market regulatory requirements.

Register means the Australian Register of Therapeutic Goods maintained under section 9A of the *Therapeutic Goods Act 1989*.

unapproved therapeutic good means a therapeutic good yet to be entered on the Register.

Part 2 – Determination that phase 0, I, II, III, pre-market pilot stage, and pre-market pivotal stage clinical trials are core R&D activities

5 Determination

Subject to section 6 of this instrument, for the purpose of Industry Innovation and Science Australia (the Board) exercising its power or performing its duty to make a finding pursuant to sections 27B, 27J or 28A of the *Industry Research and Development Act 1986,* phase 0 clinical trials, phase I clinical trials, phase II clinical trials, phase III clinical trials, pre-market pilot stage clinical trials, and pre-market pivotal stage clinical trials for an unapproved therapeutic good that are:

(a) notified pursuant to item 3 of Schedule 5A of the *Therapeutic Goods Regulations 1990* or item 2.3 of Schedule 4 to the *Therapeutic Goods (Medical Devices) Regulations 2002*; or

(b) approved pursuant to paragraphs 19(1)(b), 32CK(1)(e) or 41HB(1)(e) of the *Therapeutic Goods Act 1989*;

are core R&D activities.

6 Exclusions

None of the following will be core R&D activities for the purposes of section 5 of this instrument:

a) clinical trials of generic products;

b) clinical trials of biosimilar medicines;

c) pre-clinical trial activities or projects;

d) phase IV clinical trials;

e) market and post-market stage clinical trials;

f) activities that are within the scope of subsection 355-25(2) of the *Income Tax Assessment Act 1997*; and

g) activities that are not, or will not be, conducted in accordance with all applicable approvals, regulatory requirements, and standards that are in force at the time the phase 0 clinical trials, phase I clinical trials, phase II clinical trials, phase III clinical trials, pre-market pilot stage clinical trials, and pre-market pivotal stage clinical trials are being conducted.

Note: For the avoidance of doubt, the inclusion of activities in section 6 of this instrument does not mean these activities are not core R&D activities. Activities listed in subsubsections 6(a), (b) (c), (d) (e), (f) and (g) of this instrument may meet the definition of core R&D activities, but they do not come within the scope of section 5 of this instrument and so an R&D entity will need to demonstrate such activities meet the definition of core R&D activities independently, without the support of this instrument.

7 Part of an activity

Sections 5 and 6 of this instrument can apply to part of an activity for the purpose of the Board exercising its power or performing its duty to make a finding pursuant to sections 27B, 27J or 28A of the *Industry Research and Development Act 1986*.