Section 1: Legislation

1 Are the additional guidance documents listed in this section appropriate?

Yes

2 Are there other guidance documents relevant to the conduct of clinical trials of medicines in New Zealand that should be considered for inclusion?

Yes

3 Comments or suggestions

Comments or suggestions for section 1:
The Food and Drug Administration Safety and Innovation Act (FDASIA). Section 902 of this Act provides for a new designation - Breakthrough Therapy Designation. A breakthrough therapy is a drug:
intended alone or in combination with one or more other drugs to treat a serious or life threatening disease or condition and
preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.

New Zealand is in a unique position having world class research centres with small patient population to access. Many patients with rare diseases would be willing to enter clinical trials to allow further development of new medicines but due to the small patient population, New Zealand is often excluded from international trials. Adding in a section such as the FDA has done to the regulations would allow for innovative medicines to be developed in NZ and attract medicine development in NZ. Current research focuses on publications rather than ensuring innovative medical approaches become available to market and this limits innovative treatment opportunities for patients who are forced to travel overseas. Additionally, compounds developed in NZ are sold to foreign companies with only limited development done, which limits both the revenue coming into new Zealand but also means that potentially good New Zealand products are seen as foreign achievements.

There is already a possibility in the guideline for bioequivalence studies to be done in an expedited manner. Homegrown innovative research should be similarly expedited to encourage research in NZ

Section 2: Overview of regulation of clinical trials in New Zealand

1 Does this section adequately describe the situations when approval is required for clinical trials, and the types of approvals that are required?

Yes

2 Was the information appropriately presented?

Yes

3 Are there any changes you would like to suggest?

Yes

4 Comments or suggestions

Comments or suggestions on section 2:
With Modern Technology patients have much more information available to them regarding clinical trials. The FDA requires clinical trials to be listed on the website Clinicaltrials.gov and there is more demand from patients for access to results of clinical trials once they are available. There is no mention anywhere in section 2 regarding requirements for publication of trials, obligations of sponsor, investigator with respect to publication nor how patients should be able to access to this information.
There should be some regulation regarding this, as there is a tendancy not to publish data when there is a negative result and patients should have access to both positive and negative results of trials especially if they have participated in them.
Additionally patients in remote centres who are eager to participate in clinical trials should be able to find out easily where they are being conducted in order that they can find out more about them.

Section 3: Application for approval of a clinical trial

1 Are the roles and responsibilities of the various parties involved clearly explained?

Yes

2 Is the application process adequately described?

Yes

3 Is the sole circumstance for an abbreviated process for clinical trial approval clearly explained?

Yes

4 Comments or suggestions

Comments or suggestions on section 3:
As specified in comments on section one, an abbreviated process for clinical trial approval should be considered for NZ research centres who have innovative research and also for clinical trials for unmet medical needs.
There should be a possibility for sub investigators to participate in a trial without being part of the clinical trial site. Many long term clinical trials do not require medication to be given at every visit but do require safety follow up. this should be allowed to be done by GPS or patients own doctor, not necessarily the PI.

Section 4: Notification of clinical trial sites

1 A revised (simplified) process has been proposed for notifying clinical trial sites where subjects stay overnight as part of the investigation. Is the explanation of the requirements clear?

Yes

2 Is the revised process adequate to ensure that only trial sites with adequate access to emergency medicine facilities are used in clinical trials?

Yes

3 Are the instructions on the accompanying Clinical Trial Site Notification Form clear and easy to understand?

Yes

4 Is it clear that clinical trial applicants no longer have to notify trial sites where subjects stay overnight, and that this is the responsibility of the site manager?

Yes

5 Do you have changes to suggest that could be considered?

Yes

6 Comments or suggestions

Comments or suggestions on section 4:
The current process (followed internationally) requiring that clinical trial sites be decided prior to start of study has the following negative impacts:
-drives up clinical development costs (which has a direct follow on effect to the price of the medicine once it becomes available on the market) as clinical sites negotiate study budgets that wre often in excess of market value.
-limits accessibility of clinical trials to patients who live in big centres or who are prepared to relocate
- does not allow patients who are motivated by clinical research to participate in clinical trials if they are not seen by the Investigator at the trial site

Again due to it's size and progressive nature, New Zealand is in a unique position to change the face fo clinical trials and make them more accessible to all patients, thus limiting clinical development costs. Whilst a principal investigator would need to be determined up front, there should be flexibility in the model to allow patients from remote areas to be seen by their GP or local DHB rather than insisting that the centres be defined up front. In this model, a patient who had an untreatable disease, could request to participate in a clinical trial which yould be overseen by the prinicpal investigator but sub investigators could be at sites remote to the trial site. (eg PI could be in Auckland and patient could be in Napier and treated by local doctors under the supervision of the PI). This would require a collaborative approach from Doctors but would have multiple benefits for the patient. With modern technology access could be granted to systems and tools remote to the main investigative site.

Section 5: Good clinical practice requirements

1 Does the text in this section adequately explain what is required?

Yes

2 Are there other good clinical practice-related safety issues or safety concerns that you consider should be included in this section?

No

3 Comments or suggestions

Comments or suggestions on section 5:
GCP is an international guideline to be used in agreement with local legislation. Local legislation should always take precedence. This is adequately explained and exceptions highlighted. Listing the exceptions does run the risk that if GCP is updated this document will also need to be updated.

Section 6: Records and reporting

1 Are the responsibilities of the sponsor regarding record keeping and reporting clear?

Yes

2 Do you agree that submitting a synopsis of the final report of the clinical trial is sufficient, and that a full report does not need to be submitted unless this is asked for by Medsafe?

No

3 Do you have suggestions or recommendations to make that could be included in this section?

Yes

4 Comments or suggestions

Comments or suggestions on section 6:
Patients also have the right to know about the results of their trials. There is no requirement under this section for patients to be informed.
What if patient is treated by a medication and the study has a negative impact on their disease?
What if patient was treated by placebo and the treatment arm had remarkable results? There should be some detail as to what is expected of the applicant in these cases with respect to informing patients.
Negative results are often not published which creates a biasis in data used to get the medication on the market.

General: Layout and format of the guideline

1 Do you agree with the proposed structure of the guideline?

Yes

2 Do you have suggestions, recommendations or other information that could be included in this guideline?

No

3 Comments or suggestions

Comments or suggestions on layout and format:
The text is easy to read and straightforward.

Clinical Trial Site Notification Form

1 Does this form capture the appropriate essential information?

Yes

2 Is it obvious who should make the notification?

Yes

3 What information do you think would be useful to be published on Medsafe's list of clinical trial sites?

Comments or suggestions on what would be useful:
Types of trials being conducted. (phase, indication, research purpose) and the status of the study if it is still recruiting patients.
Responsibility for maintaining this list could be given to trial sites.
Should be able to seach list easily by geographical region.
Contact name should be person who can answer questions from potential patients.

Re-notification of clinical trial site

1 Since the self-certification process is changing to a notification procedure, would you be amenable to re-notifying your clinical trial site (if applicable) when this revised and updated guideline takes effect, so that the list of clinical trial sites is up-to-date?

Yes

2 Comments or suggestions

Comments or suggestions on re-notification:
I like the self certification process, but it is not clear enough which site is doing what types of trials. Christchurch hospital shows up several times with several different contact people for example. What is the difference or need for multiple lines?

Your details

1 Your details

Name and designation:
XXXXXXXXXXXXXXXXX

Company/organisation name (if applicable):

Address:
XXXXXXXXXXXXX
XXXXXXXXXXXXX
XXXXXXXXX

Phone number:

Email address:
XXXXXXXXXXXXXXXXXXX

2 This submission is:

from an individual or individuals (not on behalf of an organisation or in their professional capacity)

3 I am, or I represent an organisation, based in:

Other

If you selected other, please specify:

XXXXXXXXXXXXXXX

4 I am, or I represent, a:

Health professional

If you selected health professional, please indicate your type of practice:

XXXXXXXXXXXXXXXX

If you selected other, please specify:

Publishing submissions and privacy

1 Publishing submissions

You may publish this submission

2 Official Information Act responses

Remove my personal details from responses to Official Information Act requests

3 Commercially sensitive information

This submission does not contain commercially sensitive information

If your submission contains commercially sensitive information, please let us know where.:

Help us improve our consultations

1 How easy did you find using this website to make a submission?

Very easy to use

2 If you have made submissions to Medsafe or the Ministry of Health before, was making today's submission:

I haven't made a submission before

3 If there was one change you could make to the submission process, what would it be?

Top suggested change:
I have general comments on the way clinical trials should be conducted to improve the process for patients, regulators and to help minimise development costs (Which should have a positive impact on drug budgets) but as these ideas are not covered by the legislation it was difficult to determine where to put them

4 Any other comments or suggestions?

Other comments:
For many patients with unusual conditions or about to enter palliative therapy clinical trials are often something that they would like to participate in but current regulatory processes hinder patient access to these treatments. As a progressive country and world leader in research New Zealand should implement some positive changes to simplify access to clinical trials for patients and improve the drug budgets revolutionising clinical research to be a collaborative tool to improve patient care rather than an expensive development step used by the pharma industry as an excuse to charge obscene prices and drive up profits.