**Survey on Regulatory Requirements and Research Needs for Liposome Products**

**(For Regulatory Agencies)**

Thank you for your participation in our survey. Before you begin, please read the following:

In 2016, a survey was conducted among the International Pharmaceutical Regulators Forum (IPRF) Nanomedicine Working Group (NWG) members to map and exchange regulatory requirements for medicines that contain liposomal products ([Link for survey results](https://admin.iprp.global/sites/default/files/2018-09/IPRF_NWG_LiposomalResults_HC_Survey_Summary_Final.pdf)).

In Jan 2018, the IPRF and the International Generic Drug Regulators Programme (IGDRP) were consolidated to establish the International Pharmaceutical Regulators Programme (IPRP). The membership of the IPRP NWG has since expanded and currently there are 12 members and observers represented in the group ([Link for IPRP NWG Members and Observers](https://admin.iprp.global/sites/default/files/2019-12/NWG_List-MembersObservers_2019_1021.pdf)). The focus of IPRP NWG includes non-confidential information sharing, regulatory harmonization or convergence focused on nanomedicines/nanomaterials in drug products, borderline and combination products, and follow-on nanomedicines.

IPRP NWG wishes to extend the survey in order to gain an overview of the regulatory progress that the expanded regulatory membership has made with liposomal products over the past 4 years. A comparative analysis of the regulatory landscape for liposomal products will assist the working group to identify the needs of both research and standard development. The analysis will also enhance the potential for harmonization of regulatory requirements.

In this 2020 survey, some survey questions have been updated, but most remain the same for better comparison of 2016 and 2020 survey responses. In addition, there are two questions for non-regulatory stakeholders regarding regulatory research and standard needs to support liposome product development. These questions have been provided as a separate, shortened survey for non-regulatory stakeholders.

If you have any question regarding this survey, please contact the liposome survey team (Appendix 1) for clarification.

Please submit your survey responses to wenlei.jiang@fda.hhs.gov **before Sep 1st, 2020**. Thank you in advance for your cooperation.

**Appendix I: 2020 Liposome Survey Team**

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| --- | --- |
| **2020 Liposome Survey Team** | **Email** |
| **Therapeutic Goods Administration (TGA)**Anne Field | anne.field@health.gov.au |
| **European Medicines Agency (EMA)**Dolores Hernan | Dolores.Hernan@ema.europa.eu |
| **U.S. Food and Drug Administration (U.S. FDA)**Wenlei Jiang (Survey Team lead) | wenlei.jiang@fda.hhs.gov |
| **Swissmedic**Roman Leist | Roman.Leist@swissmedic.ch |
| **Federal Institute for Drugs and Medical Devices (**BfArM**)**Rene Thuermer | Rene.Thuermer@bfarm.de |

**Survey on Regulatory Requirements and Research Needs for Liposome Products**

**(For Regulatory Agencies)**

**Your organization name:**

**Contact person:**

**Contact email:**

**Contact phone:**

| **Survey Questions** | **Responses** |
| --- | --- |
| **Regulatory Scope** |
| 1. Which type of products does your agency regulate (tick as appropriate)* Medicinal products
* Human
* Veterinary
* Medical devices
* Food
* Natural health products and/or cosmetics
* Other (describe)
 |  |
| **Regulation of Liposomal Products** |
| 2. Does your agency have any pre-defined criteria (e.g. common characteristics like particle size/ components) to classify a product as a liposomal formulation? If yes, based on what criteria do you define/classify product as liposomal? Do you have any list of the characteristics? |  |
| 3. Has your agency reviewed and/or approved liposomal products?This question refers to medicinal products where the liposomes act as a drug delivery system (e.g encapsulate the drug to alter its PK/PD) and/or medicinal products where the liposomal component may have a different role (e.g. adjuvant in vaccines) * If so, what products have been approved? Could your agency provide a list of regulated products that may be considered for this survey?
* What regulatory framework is used within your agency to regulate liposomal products?
* Have you developed any specific guideline on liposomal formulations? If yes, please share.
* Has your agency consulted any external guidelines, if so, which ones?
* How does your agency regulate lipid excipients in the liposome formulation? (do you have any specific or additional requirements even if the excipients used are not novel excipients?)
 |  |
| 4. What are the non-clinical data requirements for the lipid excipient components of the liposome products? Are they similar to the data requirements for excipients of non-liposomal products? Are there any additional data requirements for the lipid components of a liposomal product?  |  |
| 5. What initiatives is your agency involved in with respect to developing/strengthening 1) regulations or guidelines associated with liposomal drug products; 2) consensus standards development? Please provide web links for any relevant regulation, guidance and standard documents.  |  |
| 6. What areas related to the regulation of liposomal drug products do you feel should be the focus of the International Pharmaceutical Regulators Programme (IPRP) Nano WG? |  |
| 7. What challenges (quality, nonclinical, clinical, and/or bioequivalence) does your agency face in the regulation and/or evaluation of liposomal drug products and how can they be addressed? |  |
| 1. Would one guidance document be able to address various liposomal formulations?

  |  |
| 1. Does your agency prefer product specific guidance? (e.g., a guidance for doxorubicin HCl liposome injections) or similar formulation specific (e.g., one guidance for all unilamellar liposomes, and another guidance for all multivesicular liposomes)?
 |  |
| 1. Does your agency have specific requirements for the post-market assessment of safety and efficacy of liposomal products?
 |  |
| 1. Have you approved or had review experiences on any lipid complex used for delivery related to (tick the ones that apply)
* Gene therapy
* Oligonucleotides
* RNA/DNA
* Peptides/Proteins
* Which regulatory pathway was used for each case?
* Do you have any additional assessment criteria for each of these products compared to conventional liposomes encapsulating small molecules?
* Gene therapy
* Oligonucleotides
* RND/DNA
* Peptides/Proteins
 |  |
| 1. Have you approved or had review experiences on any follow-on version of any innovator liposome products?

  |  |
| 1. What regulatory research needs has your agency identified for liposomes and lipid complex drug products to ensure the body of knowledge, tools and standards needed to assess the quality, safety and efficacy of these products are generated.

Compared to conventional liposomes encapsulating small molecules, are there any specific requirements for such system used in delivery related to:* gene therapy
* oligonucleotide
* RNA/DNA
* Peptides/Proteins
 |  |
| 14. Which reference materials and documentary standards do you think the standards organizations (e.g. pharmacopoeia) should develop to facilitate development of liposome and lipid complex products? (Provide top 3)  |  |
| 15. How does your agency recognize consensus standards (e.g., pharmacopeia standards, National Institute for Biological Standards and Controls (NIBSC), and standards developed by International Standard Organization (ISO), American Society of Testing and Materials (ASTM) International, and apply them into application review? |  |
|  |  |
| **Natural Health Products and/or Cosmetics involving Liposomes** |
| 16. Does your agency have specific procedure for market access of natural health products (NHP) and/or cosmetics that contain liposomes (i.e. vitamins and herbals, herbal medicines, homeopathic medicines)? If yes, what type of evidence is considered for market access? |  |
| 17. Does your agency impose additional regulatory requirements (for example, evidence of efficacy and/or more extensive product characterization for liposomal NHPs) depending upon the seriousness of the health claims made? |  |
| 18. Has your agency approved or had review experiences with any cosmetic products that contain liposomes? Which regulatory pathway was used? What are the additional assessment criteria for the liposome product compared to conventional NHP and/or cosmetics? |  |

**Survey on Regulatory Requirements and Research Needs for Liposome Products**

**(For Non-regulatory Stakeholders)**

**Your organization name:**

**Contact person:**

**Contact email:**

**Contact phone:**

Thank you for your participation in our survey. Before you begin, please read the following:

A survey was conducted among the International Pharmaceutical Regulators Forum (IPRF) Nanomedicine Working Group (NWG) members in 2016 to map and exchange regulatory requirements for medicines that contain liposomal products ([Link for survey results](https://admin.iprp.global/sites/default/files/2018-09/IPRF_NWG_LiposomalResults_HC_Survey_Summary_Final.pdf)). In Jan 2018, the IPRF and the International Generic Drug Regulators Programme (IGDRP) were consolidated to establish the International Pharmaceutical Regulators Programme (IPRP). The IPRP Nanomedicine Working Group is conducting a new survey to capture the regulatory progress for liposome products in the past 4 years from the expanded regulatory members. Some survey questions are updated but most remain the same for better comparison of 2016 and 2020 survey responses. In addition, there are 2 questions for non-regulatory stakeholders regarding regulatory research and standard needs to support liposome product development.

Please submit your survey responses via the on line link before Sep 1st, 2020. Thank you for your cooperation.

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| --- | --- |
| 1. What regulatory research needs have your organization identified for liposomes and lipid complex drug products? This will help regulatory agencies to prioritize regulatory research in this area to ensure the body of knowledge, tools and standards needed to assess the quality, safety and efficacy of the products are generated.

Are there any specific requirements for such system used in delivery related to:* gene therapy
* oligonucleotide
* RNA/DNA
* Peptides/Proteins
 |  |
| 1. Which reference materials and documentary standards do you think the standards organizations (e.g. pharmacopoeia) should develop to facilitate development of liposome and lipid complex products? (Provide top 3)
 |  |