FY 2013

PERFORMANCE REPORT TO THE PRESIDENT AND CONGRESS

for the

Biosimilar User Fee Act

Food and Drug Administration
Department of Health and Human Services

Commissioner's Report

I am pleased to present the Food and Drug Administration's (FDA) fiscal year (FY) 2013 Performance Report to Congress for the Biosimilar User Fee Act (BsUFA). On July 9, 2012, the President signed into law the Food and Drug Administration Safety and Innovation Act (FDASIA), which included the first authorization of BsUFA. BsUFA provides FDA with user fee revenue to expedite the process for the review of biosimilar biological product submissions, including applications, supplements, notifications, responses, and meeting management. The report that follows presents FDA's accomplishments for FY 2013, the first year of BsUFA.

This report details FDA's preliminary performance for FY 2013. Although FDA did not receive any original applications or supplements for biosimilar biological products in FY 2013, we did review submissions for biosimilars in development and meet with sponsors to provide advice on their biosimilar development programs. BsUFA procedural and processing goals applied to many of these submissions and meetings, including goals for procedural notifications, procedural responses, and meeting management. I can report that FDA is meeting 8 of the 11 procedural and processing performance goals that applied to these biosimilar submissions and meetings.

FDA is committed to meeting all BsUFA performance goals. We will continue to strengthen efforts to improve performance while, as always, maintaining a focus on ensuring that all biosimilar submissions are reviewed in an efficient and predictable time frame.

FDA is committed to improving the efficiency, quality, and predictability of the biosimilar review process. We are dedicated to exploring new approaches and technologies that offer high-quality, cost-effective improvements in FDA's review of biosimilar submissions.

We look forward to continued success and improvements in the biosimilar review process, made possible by BsUFA, in the coming years.

Margaret A. Hamburg, M.D.
Commissioner of Food and Drugs

Acronyms

BPD – Biosimilar Biological Product Development

BsUFA – Biosimilar User Fee Act

CBER – Center for Biologics Evaluation and Research

CDER – Center for Drug Evaluation and Research

ETASU – Elements To Assure Safe Use

FDA – Food and Drug Administration

FDASIA – Food and Drug Administration Safety and Innovation Act

FY – Fiscal Year (October 1 to September 30)

PHS – Public Health Service

REMS – Risk Evaluation and Mitigation Strategy

Executive Summary

On July 9, 2012, the President signed into law FDASIA, which included the authorization of BsUFA. BsUFA provides FDA with user fee revenue for the review of biosimilar biological product submissions to facilitate the development of safe and effective biosimilar products for the American public.

This report summarizes FDA's initial performance in meeting BsUFA goals and commitments for FY 2013, the first year of BsUFA's 5-year authorization. More detailed information on FY 2013 submissions and performance calculations, as well as definitions of key terms used in this report, are presented in the appendices.

Although FDA did not receive any biosimilar applications or supplements in FY 2013 during this first year of the BsUFA program, FDA staff expended significant effort in reviewing submissions for biosimilars in development and meeting with sponsors to discuss their biosimilar development programs. These biosimilar biological product development (BPD) meetings are critical points in the development process, enabling FDA to provide advice and clarity throughout the development stage. The meetings are also crucial to facilitating product development that leads to the submission of marketing applications.

BsUFA Performance

Because FDA had not received any biosimilar applications or supplements for review as of September 30, 2013, there are no application-related review-time goals to report in FY 2013. FDA did receive a number of submissions for biosimilars in development and requests for meetings with sponsors to discuss development programs. Performance related to these submissions and meetings is included in this report.

During FY 2013, FDA received 93 submissions for biosimilars in development and completed 81 actions related to the procedural and processing goals (i.e., procedural notifications, procedural responses, and meeting management) that applied to these submissions. In total, 11 of BsUFA's 19 procedural and processing goals applied to the FY 2013 cohort of biosimilar submissions (one procedural notification goal, one procedural response goal, and nine meeting management goals). The remaining eight goal categories (four procedural notification goals, two procedural response goals, and two meeting management goals) did not apply to the FY 2013 biosimilar submission cohort.

FDA is currently meeting or exceeding 8 of the 11 procedural and processing goals that applied to the FY 2013 biosimilar submission cohort. With 12 submissions that have not yet been acted on and have not passed the BsUFA goal due date, FDA has the potential to meet or exceed 9 of 11 procedural and processing goals that applied to the FY 2013 biosimilar submission cohort.

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Table of Contents

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Introduction

On July 9, 2012, the President signed into law FDASIA, which included the authorization of BsUFA. The Federal Food, Drug, and Cosmetic Act, as amended by BsUFA, authorizes FDA to assess and collect fees for biosimilar biological products from October 2012 through September 2017. FDA dedicates these fees to the efficient review of biosimilar submissions and to facilitating the development of safe and effective biosimilar products for the American public.

FDA's Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) are managing the biosimilar review program to ensure user fees collected under the Prescription Drug User Fee Act, the Medical Device User Fee Act, or the Generic Drug User Fee Act are not used to review applications under section 351(k) of the Public Health Service (PHS) Act. The CDER Financial Council oversees the Center's non-labor expenditures to ensure they are funded from the appropriate user fee versus non-user fee accounts. Both Centers track employee workload activities through periodic time reporting to ensure that labor costs related to the process for the review of biosimilars (versus those for the review of other human drugs or medical devices) are recorded as BsUFA work and funded from appropriate accounts.

FDA did not receive any biosimilar applications or supplements in FY 2013, so no performance data for application-related review-time goals is available in this report. FDA did receive submissions for biosimilars in development, and this report presents FDA's performance on the procedural and processing goals that applied to these submissions. As the BsUFA program matures and FDA receives more submissions, FDA looks forward to reporting on all the BsUFA performance goals.

Performance Presented in This Report

This report presents preliminary FDA performance for review of submissions received and actions taken in FY 2013 as of September 30, 2013. Final FDA performance for FY 2013 submissions will be presented in the FY 2014 BsUFA Performance Report and will include final actions for submissions still pending within the BsUFA goal date as of September 30, 2013. The following information refers to performance presented in this report.

- The following terminology is used throughout this document:
    - *Application* means a new, original application
    - *Supplement* means a supplement to an approved application
    - *Resubmission* means a resubmitted application or supplement in response to a complete response or tentative approval letter
    - *Submission* applies to all of the above

- Performance goal results are reported for each fiscal year receipt cohort (defined as

submissions filed from October 1 to September 30 of the following year). Submissions received too late to be reviewed by the end of a fiscal year are reported after FDA takes an action, or when the review-time goal period expires, whichever comes first, in subsequent years.

- Unless otherwise noted, all performance data are as of September 30, 2013.

- Preliminary performance for FY 2013 submissions is reported as the current percentage of submissions that have been reviewed within the review-time goal. The highest possible performance column shows the percent of reviews that will be completed on time if all non-overdue pending reviews are completed within goal.

- Appendix A includes the detailed preliminary performance calculations for FY 2013 including the number of submissions reviewed or addressed on time (acted on by the goal date) and the number of overdue goals (acted on past goal or currently pending but already past the goal date). Performance is presented as percent on time. Preliminary performance excludes actions pending within the BsUFA goal date.

Biosimilar Application and Supplement Types

- **Original Biosimilar Product Application** – A new application for licensure of a biological product under section 351(k) of the PHS Act.
- **Resubmitted Original Biosimilar Product Application** – A complete response to an action letter for an original application addressing all identified deficiencies.
- **Original Supplement with Clinical Data** – A request for FDA to approve a change in a biosimilar product application that has been approved, including a supplement requesting that FDA determine that the approved biosimilar meets the standards for interchangeability described in section 351(k)(4) of the PHS Act.
- **Resubmitted Supplement with Clinical Data** – A complete response to an action letter for an original supplement with clinical data addressing all identified deficiencies.
- **Manufacturing Supplement** – A request to FDA to approve a change in the manufacturing of an approved biosimilar.

Additional definitions are included in Appendix B and in the BsUFA statutory language: www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/TherapeuticBiologicApplications/Biosimilars/UCM287749.pdf

BsUFA Performance Goals and Commitments

The table below presents the goal timeline and the percentage of submissions that FDA committed to review within the goal timeline for FY 2013 through FY 2017.

FDA Performance Goal Targets

BsUFA Submission Type	Review-Time Goal	FY 13	FY 14	FY 15	FY 16	FY 17
Biosimilar Applications and Supplements						
Original Biosimilar Product Applications	10 months	70%	70%	80%	85%	90%
Resubmitted Original Biosimilar Applications	6 months	70%	70%	80%	85%	90%
Original Supplements with Clinical Data	10 months	90%	90%	90%	90%	90%
Resubmitted Supplements with Clinical Data	6 months	90%	90%	90%	90%	90%
Manufacturing Supplements	6 months	90%	90%	90%	90%	90%
Procedural Notifications						
Notification of Issues Identified During Review	74 days	90%	90%	90%	90%	90%
Notification of Planned Review Timeline	74 days	90%	90%	90%	90%	90%
Review of Proprietary Biosimilar Product Names (During BPD Phase)	180 days	90%	90%	90%	90%	90%
Review of Proprietary Biosimilar Product Names (With Application)	90 days	90%	90%	90%	90%	90%
Review of Proprietary Biosimilar Product Names (Resubmitted or Requests for Reconsideration)	60 days	90%	90%	90%	90%	90%
Procedural Responses						
Major Dispute Resolution	30 days	90%	90%	90%	90%	90%
Responses to Clinical Holds	30 days	90%	90%	90%	90%	90%
Special Protocol Assessments	45 days	70%	70%	80%	85%	90%
Meeting Management						
Meeting Requests: Initial Advisory Meeting	21 days	90%	90%	90%	90%	90%
Meeting Requests: BPD Type 1	14 days	90%	90%	90%	90%	90%
Meeting Requests: BPD Type 2	21 days	90%	90%	90%	90%	90%
Meeting Requests: BPD Type 3	21 days	90%	90%	90%	90%	90%
Meeting Requests: BPD Type 4	21 days	90%	90%	90%	90%	90%
Scheduling Meetings: Initial Advisory Meeting	90 days	70%	70%	80%	85%	90%
Scheduling Meetings: BPD Type 1	30 days	70%	70%	80%	85%	90%
Scheduling Meetings: BPD Type 2	75 days	70%	70%	80%	85%	90%
Scheduling Meetings: BPD Type 3	120 days	70%	70%	80%	85%	90%
Scheduling Meetings: BPD Type 4	60 days	70%	70%	80%	85%	90%
Provide Meeting Minutes: All Meeting Types	30 days	90%	90%	90%	90%	90%

FY 2013 Preliminary BsUFA Performance Summary

The table below presents preliminary FY 2013 BsUFA performance.

- *Review Progress* presents the number of submissions that had actions taken in FY 2013, plus submissions pending overdue as of September 30, 2013, whether or not they met the BsUFA goal date, out of all submissions received.
- *Current Performance* presents the percentage of actions that FDA completed within the review-time goal. Appendix A contains additional information on the completed reviews.
- *Highest Possible Performance* represents the scenario where all remaining non-overdue pending submissions are reviewed on time (by the BsUFA goal date).

BsUFA Submission Type	Review Progress	Performance Goal	Current Performance	Highest Possible Performance
Biosimilar Applications and Supplements				
Original Biosimilar Product Applications	0 of 0 complete	70%	NA	NA
Resubmitted Original Biosimilar Applications	0 of 0 complete	70%	NA	NA
Original Supplements with Clinical Data	0 of 0 complete	90%	NA	NA
Resubmitted Supplements with Clinical Data	0 of 0 complete	90%	NA	NA
Manufacturing Supplements	0 of 0 complete	90%	NA	NA
Procedural Notifications				
Notification of Issues Identified During Review	0 of 0 complete	90%	NA	NA
Notification of Planned Review Timeline	0 of 0 complete	90%	NA	NA
Review of Proprietary Biosimilar Product Names (During BPD Phase)	2 of 3 complete	90%	100%	100%
Review of Proprietary Biosimilar Product Names (With Application)	0 of 0 complete	90%	NA	NA
Review of Proprietary Biosimilar Product Names (Resubmitted/Requests for Reconsideration)	0 of 0 complete	90%	NA	NA
Procedural Responses				
Major Dispute Resolution	0 of 0 complete	90%	NA	NA
Responses to Clinical Holds	1 of 1 complete	90%	100%	Final
Special Protocol Assessments	0 of 0 complete	70%	NA	NA
Meeting Management				
Meeting Requests: Initial Advisory Meeting	4 of 4 complete	90%	100%	Final
Meeting Requests: BPD Type 1	0 of 0 complete	90%	NA	NA
Meeting Requests: BPD Type 2	21 of 21 complete	90%	95%	Final
Meeting Requests: BPD Type 3	6 of 6 complete	90%	100%	Final
Meeting Requests: BPD Type 4	1 of 1 complete	90%	100%	Final
Scheduling Meetings: Initial Advisory Meeting	3 of 3 complete	70%	67%	Final
Scheduling Meetings: BPD Type 1	0 of 0 complete	70%	NA	NA
Scheduling Meetings: BPD Type 2	18 of 20 complete	70%	61%	65%
Scheduling Meetings: BPD Type 3	6 of 6 complete	70%	100%	Final
Scheduling Meetings: BPD Type 4	1 of 1 complete	70%	100%	Final
Provide Meeting Minutes: All Meeting Types	18 of 27 complete	90%	89%	93%

Appendices

Appendix A: Performance Calculations

FDA did not receive any biosimilar applications or supplements in FY 2013, so no performance data for application-related review-time goals are available in this report. FDA did receive submissions for biosimilars in development, and this report describes FDA's performance on the procedural and processing goals that applied to these submissions. As the BsUFA program matures and FDA receives more submissions, FDA looks forward to reporting on all the BsUFA performance goals.

Review of Proprietary Biosimilar Product Names (During BPD Phase)

Goal: Review and act on 90 percent of submissions within 180 days

Review of Proprietary Biosimilar Product Names (During BPD Phase)	FY 2013
Total Submissions (Workload)	**3**
Pending	1
On Time	2
Overdue	0
Current Performance: % On Time	**100%**
Highest Potential Performance:	100%
BsUFA Goal: On Time Target %	**90%**
Goal Met Status:	**Currently Meeting, Pending**

Responses to Clinical Holds

Goal: Review and act on 90 percent of submissions within 30 days

Responses to Clinical Holds	FY 2013
Total Submissions (Workload)	**1**
Pending	0
On Time	1
Overdue	0
Current Performance: % On Time	**100%**
Highest Potential Performance:	Final
BsUFA Goal: On Time Target %	**90%**
Goal Met Status:	**Goal Met**

Meeting Management

Type	Review-Time Goal	Goal Target FY 13	Received*	Pending Within Goal	On Time	Overdue	Current Percent On Time
Meeting Requests							
Initial Advisory Meeting	21 Days	90%	4	0	4	0	**100%**
BPD Type 1	14 Days	90%	0	0	0	0	**NA**
BPD Type 2	21 Days	90%	21	0	20	1	**95%**
BPD Type 3	21 Days	90%	6	0	6	0	**100%**
BPD Type 4	21 Days	90%	1	0	1	0	**100%**
Scheduling							
Initial Advisory Meeting	90 Days	70%	3	0	2	1	**67%**
BPD Type 1	30 Days	70%	0	0	0	0	**NA**
BPD Type 2	75 Days	70%	20	2	11	7	**61%**
BPD Type 3	120 Days	70%	6	0	6	0	**100%**
BPD Type 4	60 Days	70%	1	0	1	0	**100%**
Meeting Minutes							
Meeting Minutes Taken	30 Days	90%	27	9	16	2	**89%**

* Not all meeting requests are granted; therefore, the number of meetings scheduled may differ from the number of meeting requests received. Not all scheduled meetings are held; therefore, the number of meeting minutes may differ from the number of meetings scheduled.

Appendix B: Definitions of Key Terms

A. The phrase *review and act on* means the issuance of a complete action letter after the complete review of a filed complete application. The action letter, if it is not an approval, will set forth in detail the specific deficiencies and, where appropriate, the actions necessary to place the application in condition for approval.

B. Goal Date Extensions for Major Amendments

1. A major amendment to an original application, supplement with clinical data, or resubmission of any of these applications, submitted at any time during the review cycle, may extend the goal date by 3 months.
2. A major amendment may include, for example, a major new clinical safety/efficacy study report; major re-analysis of previously submitted study(ies); submission of a risk evaluation and mitigation strategy (REMS) with elements to assure safe use (ETASU) not included in the original application; or significant amendment to a previously submitted REMS with ETASU. Generally, changes to REMS that do not include ETASU and minor changes to REMS with ETASU will not be considered major amendments.
3. A major amendment to a manufacturing supplement submitted at any time during the review cycle may extend the goal date by 2 months.
4. Only one extension can be given per review cycle.
5. Consistent with the underlying principles articulated in the good review management principles guidance[1], FDA's decision to extend the review clock should, except in rare circumstances, be limited to occasions where review of the new information could address outstanding deficiencies in the application and lead to approval in the current review cycle.

C. A resubmitted original application is a complete response to an action letter addressing all identified deficiencies.

D. A Biosimilar Initial Advisory Meeting is an initial assessment limited to a general discussion regarding whether licensure under section 351(k) of the PHS Act may be feasible for a particular product, and, if so, general advice on the expected content of the development program. Such term does not include any meeting that involves substantive review of summary data or full study reports.

E. A BPD Type 1 Meeting is a meeting which is necessary for an otherwise stalled drug development program to proceed (e.g., meeting to discuss clinical holds, dispute resolution meeting), a special protocol assessment meeting, or a meeting to address an important safety issue.

F. A BPD Type 2 Meeting is a meeting to discuss a specific issue (e.g., proposed study design or endpoints) or questions where FDA will provide targeted advice regarding an ongoing biosimilar biological product development program. Such term includes substantive review of summary data, but does not include review of full study reports.

[1] www.fda.gov/downloads/Drugs/.../Guidances/ucm079748.pdf

G. A BPD Type 3 Meeting is an in depth data review and advice meeting regarding an ongoing biosimilar biological product development program. Such term includes substantive review of full study reports, FDA advice regarding the similarity between the proposed biosimilar biological product and the reference product, and FDA advice regarding additional studies, including design and analysis.

H. A BPD Type 4 Meeting is a meeting to discuss the format and content of a biosimilar biological product application or supplement submitted under section 351(k) of the PHS Act.

A full list of terms related to BsUFA can be found in the BsUFA statutory language: www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/TherapeuticBiologicApplications/Biosimilars/UCM287749.pdf

Department of Health and Human Services
Food and Drug Administration

This report was prepared by FDA's Office of Planning in collaboration with the Center for Biologics Evaluation and Research (CBER) and the Center for Drug Evaluation and Research (CDER). For information on obtaining additional copies contact:

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