

**CENTER FOR DRUG EVALUATION AND  
RESEARCH**

*APPLICATION NUMBER:*

**211192Orig1s000**

**ADMINISTRATIVE and CORRESPONDENCE  
DOCUMENTS**



IND 119341

**MEETING MINUTES**

Agios Pharmaceuticals, Inc.  
Attention: Shane A. McGann, PharmD, RPh  
Manager, Regulatory Affairs  
88 Sidney Street  
Cambridge, MA 02139

Dear Dr. McGann:

Please refer to your Investigational New Drug Application (IND) submitted under section 505(i) of the Federal Food, Drug, and Cosmetic Act for AG-120.

We also refer to the meeting between representatives of your firm and the FDA on May 25, 2016. The purpose of the meeting was to obtain guidance on the

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A copy of the official minutes of the meeting is enclosed for your information. Please notify us of any significant differences in understanding regarding the meeting outcomes.

If you have any questions, call Laura Wall, Regulatory Project Manager at (301) 796-2237.

Sincerely,

*{See appended electronic signature page}*

Donna Przepiorka, MD, PhD  
Acting Clinical Team Leader  
Division of Hematology Products  
Office of Hematology and Oncology Products  
Center for Drug Evaluation and Research

Enclosure:  
Meeting Minutes



**FOOD AND DRUG ADMINISTRATION**  
**CENTER FOR DRUG EVALUATION AND RESEARCH**

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**MEMORANDUM OF MEETING MINUTES**

**Meeting Type:** Type B  
**Meeting Category:** Pre-Phase 3

**Meeting Date and Time:** May 25, 2016 from 2:00 PM to 3:00 PM (ET)  
**Meeting Location:** White Oak Building 22, Conference Room: 1313

**Application Number:** IND 119341  
**Product Name:** AG-120  
**Proposed Indication:** Treatment of patients with acute myelogenous leukemia (AML) harboring an isocitrate dehydrogenase-1 (IDH1) mutation:

- As a single agent for the treatment of adult patients with relapsed or refractory (R/R) AML

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**Sponsor/Applicant Name:** Agios Pharmaceuticals, Inc.

**Meeting Chair:** Donna Przepiorka, MD, PhD, Acting Clinical Team Leader  
**Meeting Recorder:** Laura Wall, MS, Regulatory Project Manager

**FDA ATTENDEES**

Division of Hematology Products (DHP)

Ann Farrell, MD, Division Director  
Albert Deisseroth, MD, PhD, Clinical Team Leader  
Donna Przepiorka, MD, PhD, Acting Clinical Team Leader  
Pat Dinndorf, MD, Clinical Reviewer  
Ashley Ward, MD, Clinical Reviewer,  
Laura Wall, MS, Regulatory Project Manager

Office of Clinical Pharmacology, Division of Pharmacometrics

Olanrewaju Okusanya, PharmD, MS, Clinical Pharmacology Reviewer

Office of Biostatistics, Division of Biometrics V

Lei Nie, PhD, Team Leader  
Kallappa Koti, PhD, Statistical Reviewer

Division of Hematology Oncology Toxicology (DHOT)

Christopher Sheth, PhD, Supervisory Pharmacologist/Toxicologist  
Matthew Thompson, PhD, MPH, Pharmacology/Toxicology Reviewer

Office of Translational Sciences  
Sarah Dorff, PhD, Genomics Reviewer

**SPONSOR ATTENDEES**

Chris Bowden, MD, Agios, Chief Medical Officer  
Sam Agresta, MD, MPH & TM, MS CI & TR, Agios, Vice-President, Clinical Development  
Ann Cahill, PA, Agios, Senior Director, Clinical Development  
Eyal Attar, MD, Agios, Medical Director, Clinical Development  
Meredith Goldwasser, ScD, Agios, Senior Director, Head of Biometrics and Data Management  
Hua Liu, PhD, Agios, Associate Director of Biostatistics  
Jacqueline Cinicola, MS, Agios, Senior Director, Regulatory Affairs  
Shane McGann, PharmD, RPh, Agios, Manager, Regulatory Affairs  
Annie Estrella, MS, Agios, Director, Head of Medical Writing  
Katharine Yen, PhD, Agios, Director, Clinical Science  
Paul McNulty, Celgene, Executive Director, Global Regulatory Affairs  
Krishnan Viswanadhan, PharmD, MBA, Celgene, Global Project Leadership, Alliance Partner

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**1.0 BACKGROUND**

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### **3.0 OTHER IMPORTANT MEETING INFORMATION**

#### **PREA REQUIREMENTS**

Under the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c), all applications for new active ingredients (which includes new salts and new fixed combinations), new indications, new dosage forms, new dosing regimens, or new routes of administration are required to contain an assessment of the safety and effectiveness of the product for the claimed indication(s) in pediatric patients unless this requirement is waived, deferred, or inapplicable.

Because this drug product for this indication has an orphan drug designation, you are exempt from these requirements. Please include a statement that confirms this finding, along with a reference to this communication, as part of the pediatric section (1.9 for eCTD submissions) of your application. If there are any changes to your development plans that would cause your application to trigger PREA, your exempt status would change.

## **DATA STANDARDS FOR STUDIES**

Under section 745A(a) of the FD&C Act, electronic submissions “shall be submitted in such electronic format as specified by [FDA].” FDA has determined that study data contained in electronic submissions (i.e., NDAs, BLAs, ANDAs and INDs) must be in a format that the Agency can process, review, and archive. Currently, the Agency can process, review, and archive electronic submissions of clinical and nonclinical study data that use the standards specified in the Data Standards Catalog (Catalog) (See <http://www.fda.gov/forindustry/datastandards/studydatastandards/default.htm>).

On December 17, 2014, FDA issued final guidance, *Providing Electronic Submissions in Electronic Format--- Standardized Study Data* (<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM292334.pdf>). This guidance describes the submission types, the standardized study data requirements, and when standardized study data will be required. Further, it describes the availability of implementation support in the form of a technical specifications document, Study Data Technical Conformance Guide (Conformance Guide) (See <http://www.fda.gov/downloads/ForIndustry/DataStandards/StudyDataStandards/UCM384744.pdf>), as well as email access to the eData Team ([cdere-data@fda.hhs.gov](mailto:cdere-data@fda.hhs.gov)) for specific questions related to study data standards. Standardized study data will be required in marketing application submissions for clinical and nonclinical studies that start on or after December 17, 2016. Standardized study data will be required in commercial IND application submissions for clinical and nonclinical studies that start on or after December 17, 2017. CDER has produced a [Study Data Standards Resources](#) web page that provides specifications for sponsors regarding implementation and submission of clinical and nonclinical study data in a standardized format. This web page will be updated regularly to reflect CDER's growing experience in order to meet the needs of its reviewers.

Although the submission of study data in conformance to the standards listed in the FDA Data Standards Catalog will not be required in studies that start before December 17, 2016, CDER strongly encourages IND sponsors to use the FDA supported data standards for the submission of IND applications and marketing applications. The implementation of data standards should occur as early as possible in the product development lifecycle, so that data standards are accounted for in the design, conduct, and analysis of clinical and nonclinical studies. For clinical and nonclinical studies, IND sponsors should include a plan (e.g., in the IND) describing the submission of standardized study data to FDA. This study data standardization plan (see the Conformance Guide) will assist FDA in identifying potential data standardization issues early in the development program.

Additional information can be found at <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/ucm248635.htm>.

For general toxicology, supporting nonclinical toxicokinetic, and carcinogenicity studies, CDER encourages sponsors to use Standards for the Exchange of Nonclinical Data (SEND) and submit sample or test data sets before implementation becomes required. CDER will provide

feedback to sponsors on the suitability of these test data sets. Information about submitting a test submission can be found here:

<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/ucm174459.htm>.

## **LABORATORY TEST UNITS FOR CLINICAL TRIALS**

CDER strongly encourages IND sponsors to identify the laboratory test units that will be reported in clinical trials that support applications for investigational new drugs and product registration. Although Système International (SI) units may be the standard reporting mechanism globally, dual reporting of a reasonable subset of laboratory tests in U.S. conventional units and SI units might be necessary to minimize conversion needs during review. Identification of units to be used for laboratory tests in clinical trials and solicitation of input from the review divisions should occur as early as possible in the development process. For more information, please see the FDA website entitled, [Study Data Standards Resources](#) and the CDER/CBER Position on Use of SI Units for Lab Tests website found at <http://www.fda.gov/ForIndustry/DataStandards/StudyDataStandards/ucm372553.htm>.

## **NEW PROTOCOLS AND CHANGES TO PROTOCOLS**

To ensure that the Division is aware of your continued drug development plans and to facilitate successful interactions with the Division, including provision of advice and timely responses to your questions, we request that the cover letter for all new phase 2 or phase 3 protocol submissions to your IND or changes to these protocols include the following information:

1. Study phase
2. Statement of whether the study is intended to support marketing and/or labeling changes
3. Study objectives (e.g., dose finding)
4. Population
5. A brief description of the study design (e.g., placebo or active controlled)
6. Specific concerns for which you anticipate the Division will have comments
7. For changes to protocols only, also include the following information:
  - A brief summary of the substantive change(s) to the protocol (e.g., changes to endpoint measures, dose, and/or population)
  - Other significant changes
  - Proposed implementation date

We recommend you consider requesting a meeting to facilitate discussion of multiple and/or complex issues.

### **4.0 ISSUES REQUIRING FURTHER DISCUSSION**

None

### **5.0 ACTION ITEMS**

None

## **6.0 ATTACHMENTS AND HANDOUTS**

Attached are Agios' responses to the Agency's preliminary comments and the slides presented at the meeting. The responses were received via e-mail on May 23, 2016, and the slides were received via e-mail on May 24, 2016.

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**This is a representation of an electronic record that was signed electronically and this page is the manifestation of the electronic signature.**  
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/s/  
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DONNA PRZEPIORKA  
06/08/2016