



**Tracking Form for Applicants for New Technology Add-on Payments
under the Acute Inpatient Prospective Payment System (IPPS) for
Federal Fiscal Year (FY) 2010**

1. Technology Name:

Clofarabine

2. Manufacturer Name:

Genzyme Oncology

3. Trade Brand of Technology:

CLOLAR[®] (clofarabine) injection

4. Brief Description of Service or Device:

CLOLAR is an intravenously infused chemotherapeutic agent used in the treatment of acute leukemia.

Newness Criterion

Note: To qualify for a new technology add-on payment, the technology or service must not be reflected in the data used to establish the diagnosis related groups (DRGs).

5. Date of Food and Drug Administration (FDA) approval (or expected approval) for the device or service:

CLOLAR received FDA premarket approval (PMA) on 28 December 2004 for pediatric patients 1 to 21 years old with relapsed or refractory acute lymphoblastic leukemia after at least two prior regimens. Supplemental FDA approval is pending for the treatment of adult patients with acute myeloid leukemia. The expected approval date for the expanded indication is proprietary information contained within the full application.

6. Was the product available on the market immediately after FDA approval? If not, please provide the date that the medical service or technology came on the market (i.e. first sales or availability) and an explanation for any delay (i.e. manufacturing issues, shelf life concerns or other reasons).

(For the complete application requirements, please see the instructions at http://www.cms.hhs.gov/AcuteInpatientPPS/08_newtech.asp#TopOfPage--).

Note: The information provided on this tracking form will be made publicly available.

Yes. CLOLAR was immediately available after FDA approval for pediatric patients with relapsed or refractory acute lymphoblastic leukemia. CLOLAR is not yet FDA approved for patients with acute myeloid leukemia.

7. Does the technology have an International Classification of Diseases, Ninth Revision, Clinical Modification (ICD-9-CM) procedure code(s) or is an application pending?

CLOLAR does not currently have an existing ICD-9-CM procedure code. An application for this code will be filed with the ICD-9-CM Coordination and Maintenance Committee on or before December 1, 2008.

a. If yes, please provide the ICD-9-CM procedure code(s) used to identify the clinical procedure(s) with which the medical service and technology is used.

b. If there is no existing ICD-9-CM code that captures this new technology, please indicate whether you will be applying for a new code. (Refer to http://www.cms.hhs.gov/ICD9ProviderDiagnosticCodes/01_overview.asp for more information.) We note that, if the product were to receive add-on payment status approval, it would need to be distinctly identifiable by ICD-9-CM code(s) in the MedPAR claims data in order to receive add-on payment.

See above.

8. Have you submitted an application for outpatient pass-through payments under the Medicare outpatient prospective payment system? If so, please provide the tracking number or, if it was approved, please provide the date of approval. (Please refer to http://www.cms.hhs.gov/HospitalOutpatientPPS/04_passthrough_payment.asp for more information.)

CLOLAR was designated as eligible for pass-through status under the Medicare outpatient prospective payment system effective July 1, 2005, following its FDA approval for pediatric patients with acute lymphoblastic leukemia. No application has been submitted for pass-through status for CLOLAR for treatment of adult myeloid leukemia.

Cost Criterion

Note: To qualify for a new technology add-on payment, the technology or service must result in average charges for cases using the technology in excess of the lesser of 75 percent of the standardized amount increased to reflect the difference between costs and charges or 75 percent of 1 standard deviation beyond the geometric mean standardized charge for all cases in the DRGs to which the new technology is assigned. Table 10 from the annual final rule lists the thresholds by DRG. The most recent version of Table 10 can be downloaded at: http://www.cms.hhs.gov/AcuteInpatientPPS/08_newtech.asp
Provide the following information to demonstrate the technology or service meets the criterion.

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9. What is the anticipated average standardized charge per case involving this new technology? For details how to standardize charges please refer to the technical appendix of the application form.

The anticipated average standardized charge per case is \$135,149. Information on the data and methodology used to calculate the per case charge is contained in the full application.

10. What is the total estimated cost per case for the service or technology (this will include all costs involved in the case, including the cost of the service or device)? What is the cost of the technology per patient? Please provide a breakdown how the cost of the technology is calculated (i.e. **Drugs**- Average dosage or number of units per patient (ml/kg/hr); **Devices**- breakdown of the cost of all components used in the new technology, clearly showing which components are the “new” ones).

Proprietary information contained in full application.

11. List the diagnosis-related groups (DRGs) to which cases involving this new technology will most likely be assigned.

- MS-DRG 837 Chemo with Acute Leukemia with MCC
- MS-DRG 838 Chemo with Acute Leukemia with CC
- MS-DRG 839 Chemo with Acute Leukemia without CC/MCC

12. What is the anticipated volume of Medicare cases involving the use of this technology in FY 2010 (by DRG)?

Proprietary information contained in full application.

Clinical Improvement Criterion

Note: To qualify for a new technology add-on payment, the technology or service must represent a substantial clinical improvement over existing technologies or services.

13. Please provide a short synopsis of the following clinical issues added to the new technology. Use the regular application to submit full details.

- a. Briefly describe how the new service or technology represents a substantial clinical improvement over existing services or technologies.

CLOLAR provides a new treatment option for older adult patients with AML who are likely to have poor treatment outcomes if given conventional therapy. A high proportion of older patients with acute myeloid leukemia (AML) present with poor prognostic factors and resistance to available chemotherapy agents, that put them at risk for poor treatment outcomes, such as low complete remission rates and high 30 day induction mortality, along with a short duration of remission, as compared with younger patients with newly diagnosed disease.

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Poor tolerability and questionable treatment benefit have left older adult patients with AML often without any treatment options.

In recent studies, CLOLAR has demonstrated acceptable tolerability, and effectiveness at inducing high remission rates in older patients with AML with one or multiple adverse risk factors. In addition, its use has been associated with encouraging improvements in 30-day mortality rates. The improved clinical outcomes achieved by single agent CLOLAR in these patients represents a significant clinical advancement for patients with one or more unfavorable prognostic factors who typically do not benefit from conventional induction chemotherapy.

b. List all published peer-review articles relevant to the new service or technology.

- Kantarjian H, Gandhi V, Cortes J, et al. Phase 2 clinical and pharmacologic study of CLOLAR in patients with refractory or relapsed acute leukemia. *Blood*. 2003;102:2379-2386.
- Faderl S, et al. CLOLAR and cytarabine combination as induction therapy for acute cytarabine (ara-C) in relapsed and refractory acute leukemias. *Blood* 2005; 105; 940-947.
- Faderl S, Ravandi F, Ferrajoli A, et al. CLOLAR and CLOLAR plus low-dose cytarabine (ara-C) as induction therapy for patients (pts) ≥ 60 years with newly diagnosed acute myeloid leukemia (AML). *Blood*. 2005; 106: 786a.
- Faderl S, et al. CLOLAR and cytarabine combination as induction therapy for acute myeloid leukemia (AML) in patients 50 years of age or older. *Blood*. 2006; 108: 45-51.
- Burnett AK, Baccarani M, Johnson P, et al. CLOLAR as first-line treatment of elderly (>65 yrs) AML patients with an unfavorable cytogenetic profile who are unsuitable for standard treatment. *Haematologica*. 2006; 91 (s1): 45.
- Agura ED, Berryman R, Cooper B, et al. Phase II study of CLOLAR and cytosine arabinoside in adult patients with relapsed AML and in elderly patients with untreated AML who are at high risk of anthracycline toxicity. *J Clin Oncol*. 2007; 25: 372s.
- Faderl S, et al. A randomized study of clofarabine versus clofarabine plus low-dose cytarabine as frontline therapy for patients age ≥ 60 years with acute myeloid leukemia and high-risk myelodysplastic syndrome. *Blood*. 2008; 12; 1638.
- H. P. Erba, et al. CLOLAR in previously untreated older adult patients with acute myelogenous leukemia (AML) unlikely to benefit from standard induction chemotherapy. *Am. Soc. Clin. Onc.* 2008; Poster.

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