Medicare Off-Label Approval Checklist

☐ A statement from the physician on the accepted standard of medical practice for the diagnosis and the medical circumstances of this case that the physician believes justifies the use of the drug.

☐ Clinical research literature that appears in at least two Phase III clinical trials that definitively demonstrates the safety and effectiveness; OR, at least two Phase II clinical trials with reasonably large patient samples showing consistent results of safety and efficacy.

☐ Clinical research that appears in peer reviewed medical literature.

☐ Published recommendations from specialty societies or in other authoritative evidence-based guidelines supporting a use that is an accepted standard of medical practice.

For supporting clinical research, provide the number of research subjects:

☐ location of the centers where the research was conducted

☐ response rate (Ex. evidence of significant decrease in tumor size or reduction in tumor related symptoms. Stabilization is not considered a response to therapy.)

☐ effect on key status and survival indications, and

☐ study design.

Attach any supporting literature from the following publications:

☐ American Journal of Medicine:

☐ Annals of Internal Medicine; Annals of Oncology;

☐ Annals of Surgical Oncology;

☐ Biology of Blood and Marrow Transplantation;

☐ Blood;

☐ Bone Marrow Transplantation; British Journal of Cancer; British Journal of Hematology; British Medical Journal;

☐ Cancer;

☐ Clinical Cancer Research; Drugs;
After such evidence is received, Medicare will, with appropriate help of specialty specific consultants as indicated, make a coverage determination for the non-FDA approved indication (off-label use) of the drug or biological.

In those cases when Medicare does not approve off-label use of the drug or biological in question an ABN may be necessary. (http://www.cms.hhs.gov/BNI/02_ABNGABNL.asp)

Once the checklist and relevant data is gathered please forward to the:

Outpatient Registration Manager, YNHHS at the PCC, Clinical Registration, Room 16E.

Submission Information

Once the checklist is completed and the supporting data is provided, YNHHS will follow the below submission information to:

Medicare: Paul Deutsch, MD
Medical Director, MAC J-13
National Government Services
P.O. Box 4767
Syracuse, NY 13221-4767
Source: CMS Local Coverage Determination (National Government Services) Drugs and Biologicals, coverage of, for Label and Off-Label uses (L25820).