Local Excision for Rectal Cancer

John M. Skibber, MD, Houston, Texas

Key words
Rectal carcinoma, local excision, sphincter preservation, radical resection, adjuvant therapy, surgical techniques

Abstract
Local excision can be a definitive surgical procedure for some early cancers of the rectum, and it is an appealing technique in many ways. It reduces overall surgical trauma and can assure sphincter preservation. It is also associated with low morbidity. However, this technique has a number of limitations also. This article discusses the results of local excision for rectal carcinoma, including histologic features that impact survival and local recurrence-free rates, the importance of careful patient selection, and the potential role of adjuvant and salvage therapies. Technical considerations and alternative therapies are also discussed. (JNCCN 2005;3:531–539)

The treatment goal for rectal carcinoma is cure with local control of disease while maintaining quality of life. Surgery is an important treatment option, and survival rates in patients with T1 rectal carcinomas treated with local excision or radical resection are 90% to 100%. Adequate surgical removal of the tumor is the major treatment factor affecting local control and cure,1 but the biology of a particular patient’s tumor, including venous or lymphatic involvement, is also an important factor in overall outcome.

The principles of surgical management of rectal cancer are removal of the primary tumor with adequate margins of normal tissue, treatment of the draining lymphatics, and restoration of function. Factors that can help identify patients most likely to benefit from local excision include small tumors, absence of lymphatic and vascular invasion, well- or moderately differentiated tumor, and absence of clinical or radiologic evidence of enlarged lymph nodes (Table 1). Appropriate adjuvant therapies can enhance local control, reduce systemic recurrence, and increase organ preservation.2,5

This article discusses the results of local excision for rectal carcinoma, including histologic features that impact survival and local recurrence-free rates, the resulting importance of careful patient selection, and the potential role of adjuvant and salvage therapies. Important technique considerations and alternative therapies are also discussed.

Benefits and Risks of Local Excision
The use of local excision provides a number of advantages, including minimizing surgical stress, allowing sphincter preservation, and reducing mortality and complication rates. These issues, especially sphincter preservation, can be important for maintaining patient quality of life. However, these advantages, although considerable, must also be weighed against potential disadvantages, such as the possibility of increased local recurrence or lessened survival compared with standard surgical procedures, such as low anterior resection, proctectomy and colo-anal anastomosis, or abdominoperineal resection (APR).

Several studies indicate an improved cancer-free survival for T1 cancers treated using standard oncologic resection compared with local excision,12 including in patients with mid- and low-rectum T1 tumors.13 Despite these risks, local excision alone is considered a reasonable treatment for some T1-T2 carcinoma of the rectum if the tumor meets appropriate selection criteria, but decision-making can be complicated by the facts that sphincter preservation is usually possible with more radical surgical procedures and that local and distant cancer recurrences can occur with either type of treatment, even with early rectal cancers.

Results to Date
Many studies, mostly retrospective and single-institution, have examined the results of local excision alone in the management of T1-2 rectal cancer to determine optimal strategies for using this technique. Curative surgical
treatment for rectal cancer depends on providing adequate tumor-free margins and treating the lymph nodes draining the tumor site. Local excision alone meets only the first requirement, however, and local excision procedures preclude lymph node excision. Therefore, patients with proven or suspected lymph involvement are not appropriate candidates for local excision.

Willet et al. (new ref; Cancer 1994) retrospectively reviewed results for 125 patients with T1-2 rectal cancer who underwent local excision (56 patients) or APR (69 patients) between 1962 and 1991. For 28 patients with histologic features considered favorable (well or moderately differentiated tumor and no venous or lymph involvement) undergoing local excision alone, the authors found 5-year recurrence-free and local control rates of 87% and 96%, respectively. Conversely, the results for the same procedure in 28 patients with histologic features considered unfavorable (poorly differentiated tumor or venous or lymphatic involvement) were 57% and 68%, respectively. Lymph Node Metastases: A major factor predicting patient survival and perirectal lymph node metastasis is the depth of penetration of the primary tumor. In 1966, Morson reported that lymphatic metastasis arose from 10% of tumors confined to the submucosa, 12% of tumors invading the muscularis propria, and 58% of tumors extending beyond the bowel wall. T2 rectal carcinoma also predicts a higher incidence of lymphatic metastasis. Studies of tumors treated using radical resection have found incidence rates of lymphatic metastasis of 12% for T1 tumors and 22% for T2 tumors. Morson concluded that the risk of lymph node metastasis is 10% to 30% for T2 rectal carcinomas.

The incidence of lymphatic metastasis was also increased in the presence of lymphatic or blood vessel invasion or poorly differentiated tumors. Physicians should also note, however, that many of the classic criteria can be unreliable predictors of lymphatic involvement. Nelson et al. reported that 29% of patients with lesions smaller than 2 cm in diameter had evidence of lymph node metastasis. Thus, selection for local excision based on the classic indications may lead to inadequate treatment of a significant number of patients with lymphatic involvement.

Local Recurrence: Studies also show higher local recurrence rates for tumors involving the muscularis propria that are treated with local excision alone, possibly because of the failure to treat lymphatic metastases. Both T1 and T2 tumors have shown significant local recurrence rates after local excision alone, especially when followed up for 5 or more years. In a review of 16 published series with reasonable follow-up times in which this approach was used, Graham et al. found a combined local recurrence rate after local excision alone of 5% (range, 0%-12%) for T1 lesions and 18% (range, 8%-27%) for T2 lesions. In their long-term study, Paty et al. found 10-year local recurrence rates of 17% for T1 rectal tumors and 24% for T2, with or without postoperative radiation. Their survival rates were 78% to 82% with excision alone in these patients. Other researchers specifically evaluating outcomes for patients with T1 rectal cancer treated by local excision alone, have found local recurrence rates as high as 29% at 5 years.

Pretreatment Locoregional Staging for Rectal Cancer

Patient selection is paramount to achieving good outcomes and requires understanding the limitations of locoregional excision techniques and the biology of T1-T2 rectal cancer. The criteria used to select patients for local excision are intended to make a negative-margin, full-thickness local excision technically feasible and to ensure a low risk of lymph node metastasis (Table 1).

Pretreatment locoregional staging is important in the evaluating whether patients are appropriate candidates for local excision. Physical assessment, computed tomography (CT), magnetic resonance imaging (MRI), and endorectal ultrasonography (EUS) can be helpful in determining the depth of tumor penetration into the rectal wall and whether enlarged lymph nodes are present. Digital rectal examination has been found to be poor for preoperative staging. Table 2 shows the accuracy of EUS, MRI, and CT for the T and N staging of rectal cancer preoperatively. Overall, EUS appears to be the most accurate way to

<table>
<thead>
<tr>
<th>Table 1 Indications for the Local Excision of Rectal Cancer</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Tumor &lt; 3 cm in greatest dimension.</td>
</tr>
<tr>
<td>• Involves only the submucosa or superficial muscularis.</td>
</tr>
<tr>
<td>• Favorable pathologic grade.</td>
</tr>
</tbody>
</table>

stage the depth of penetration, although MRI with special coils can also be highly accurate.

Nodal staging is less accurate with all these modalities. In general, overstaging is more common than understaging, and the addition of fine-needle aspiration to EUS does not seem to greatly improve the accuracy of nodal staging.\textsuperscript{17} In a prospective study, FNA-guided EUS had the most potential in patients with early T-stage disease being considered for local excision.\textsuperscript{32} Lymph node staging using positron emission tomography (PET) scanning appears to be limited even before changes induced by chemoradiation, with a sensitivity between 22% and 29%.\textsuperscript{33,34}

**Techniques**

Despite concerns regarding recurrence, local excision can be effective in treating selected early low rectal cancers, and can be used as curative therapy for patients who have superficial tumors (Table 3). Local excision is performed in a full-thickness manner, meaning that the deep plane of dissection should include the perirectal fat. The choice of technique for local excision is dictated by tumor characteristics such as size and circumferential involvement as well as on the surgeon’s ability to expose the tumor and control the margins of excision. Transanal excision is the most common method of local excision and is generally associated with less morbidity. Posterior approaches have the advantage of better exposure of larger lesions but are associated with a higher rate of fistula formation and the potential for tumor seeding of the posterior wound. Whichever method is selected, the full-thickness excision must have at least 1-cm margins of normal tissue surrounding the tumor; an inadequate margin predicts failure.\textsuperscript{38} Piecemeal or submucosal excision is not considered adequate surgical treatment of invasive rectal cancer, and fragmentation of the tumor is also associated with an increased incidence of local recurrence.\textsuperscript{39}

If the lesion cannot be adequately resected using local excision, a more standard locoregional surgical approach such as low anterior resection, proctectomy with colo-anal anastomosis, or APR should be used. In a curative case, the patient should be counseled to consider local excision as a form of definitive biopsy that may indicate the need for additional surgery, especially when transmural penetration or adverse histologic characteristics are found in the local excision specimen.

In patients with T2 carcinomas of the rectum, the risk of lymph node metastasis in the mesorectum should be addressed using either resection of the mesorectum during proctectomy or, if a local excision is performed, the addition of concomitant chemoradiation and radiation therapy to reduce the incidence of pelvic recurrence.

**Transanal Endoscopic Microsurgery**

Transanal endoscopic microsurgery (TEM), in which either submucosal excision (for adenomas) or full-thickness excision (for invasive carcinomas) is
performed through a surgical rectoscope, has recently emerged as another option for the local treatment of rectal cancer. It has also been successfully performed with low complication rates. Proponents of this approach claim that it allows improved exposure over the transanal approach without the risk of fecal fistula or sphincter dysfunction of the posterior or trans-sphincteric proctotomy. In a recent series, local recurrence occurred in 2 of 16 patients (12.5%) with T1 lesions undergoing TEM. However, the authors of this series thought that TEM alone was not appropriate treatment for T2 lesions, and further follow-up and experience are required to establish a role for TEM.

**Preoperative Chemoradiation**

Preoperative chemoradiation therapy has been used to improve local control and survival in patients who cannot tolerate or decline radical resection. Despriet et al. reported results in 25 patients with rectal cancer treated with preoperative external radiation therapy (35 Gy) followed by local excision and brachytherapy. Local recurrence developed in 5 patients and 1 developed distant metastases. Mohiuddin et al. reported results in 14 patients who underwent preoperative radiation therapy (45 Gy) followed by a full-thickness excision; local recurrence developed in 3 patients. In a series of 10 patients with T2-T3 primary tumors and preoperative chemotherapy followed by local excision, Ruo et al. approach noted short-term local control and a 78% 2-year survival. These results suggest that preoperative use of chemotherapy and radiation therapy to downstage the disease and permit a more satisfactory local excision for patients with T2-T3 disease may be feasible.

**Adjuvant Therapy**

The clinically significant rate of local recurrence with local excision, especially compared with lower rates of local recurrence in historical series of similar patients treated with APR (0%-10%), has driven multiple studies examining the use of both postoperative radiotherapy and postoperative chemoradiation after local excision in selected patients. In one of the first series with an adequately long follow-up, Bailey et al. reported their experience with local excision between 1978 and 1988. Of the 65 study patients, 34 (54%) received postoperative radiotherapy, and 2 of those (5.9%) had local recurrences. The crude 5-year survival rate in this series was 74.3%, and the 5-year disease-specific survival rate was 90.3%. This study provided some of the first indirect evidence regarding the long-term efficacy of adjuvant radiotherapy as well as showing the need for long-term follow-up to determine accurate rates of survival and local recurrence.

In a similar report from the Massachusetts General and Emory University Hospitals, 52 patients were treated with local excision alone, while 47 patients were given postoperative adjuvant radiotherapy. Although the patients chosen to receive postoperative radiotherapy were at higher risk of local failure because they had higher stage lesions than the patients treated with local excision alone (70% T2 vs. 15% T2, respectively), 5-year local recurrence-free survival (LRFS) and disease-free survival (DFS) rates were significantly better in patients who received adjuvant therapy (LRFS, 10% vs. 28%; DFS, 74% vs. 66%). The median follow-up in this study was 51 months. The authors also reported trends, although not statistically significant, toward improved survival and less local recurrence in a subgroup of patients given 5-fluorouracil-based chemotherapy in addition to adjuvant radiotherapy. The authors concluded that adjuvant chemoradiation should be offered to all patients with T2 disease undergoing local excision as well as all those with T1 disease with high-risk histologic features (advanced grade or lymphatic/vascular invasion). Another study showed similar trends toward improvement in local control with postoperative radiation (36% to 9%) for T2 rectal cancers treated with local excision, while the beneficial effect for T1 cancers was not seen.

Although the role of adjuvant therapies in improving outcomes for T1-2 rectal cancer treated with local excision is still being determined, a caveat is that blood vessel or lymphatic invasion is a significant predictor of lymph node involvement and poor survival. In such cases, either standard surgical therapy involving total mesorectal excision or, if the patient refuses or cannot tolerate standard surgical therapy, the use of adjuvant therapy after local excision should be considered.

Another patient group for whom adjuvant therapy should be considered are those with T3 disease. Patients with T3 carcinoma of the rectum are considered at high risk for local recurrence and have experienced local recurrent rates as high as 75% after local excision, even with the use of postoperative radiation. However, patients with T3 tumors who are...
selected for local excision are often poor candidates for more extensive surgical procedures because of comorbid disease, and therefore have limited alternative options.

Conversely, a prospective series from The University of Texas M. D. Anderson Cancer Center reported excellent local control rates for 46 patients treated with local excision and postoperative chemotherapy or radiation therapy. This group included patients with T3 tumors who were medically compromised or refused standard therapy. All tumors were less than 5 cm from the anal verge and smaller than 4 cm. All patients underwent negative-margin, full-thickness excisions via a transanal, trans-sphincteric, or posterior approach to the rectum. Postoperative radiation therapy (53 Gy) was given through posterior and two lateral fields. 5-Fluorouracil was given concurrently during radiotherapy to 8 patients with T3 lesions. The overall survival rate at 3 years was 93%.

Table 4 shows the pattern of treatment failure by the American Joint Committee on Cancer (AJCC) T stage. An update of the M. D. Anderson Cancer Center experience with local excision seems to support these findings, with 4-year LRFS rates of 9%, 80%, and 73% for T1, T2, and T3 tumors, respectively. Multivariate analysis found that only tumor stage and margin status were independent predictors for local recurrence.

Results from the Cancer and Leukemia Group B
Perhaps the best data regarding the current approach to local excision for T1-2 rectal cancer come from the initial results of a Cancer and Leukemia Group B (CALGB) prospective phase II trial. Selection criteria for local excision may be poorly defined in some single-institution studies. This study, however, had clear entry criteria used prospectively: mobile tumors confined to the rectal wall (T1-T2), less than 4 cm in size, less than 40% of the bowel wall circumference in size, and with no evidence of lymph node involvement. Patients were registered after a negative-margin, full-thickness local excision was performed. Patients with T1 tumors received no further treatment, and patients with T2 tumors received adjuvant chemoradiation therapy.

Recognizing the impracticality of using a randomized clinical trial to directly compare local excision with more radical locoregional procedures, the authors conducted this trial with 3 main goals:
1. To determine whether survival of patients with T1 and T2 adenocarcinoma of the rectum who are treated using limited, sphincter-sparing surgery is comparable to that of historical controls treated using APR
2. To assess the locoregional recurrence rate of rectal cancer as a function of tumor stage
3. To assess the toxicity of combining limited, sphincter-sparing surgery with post-excisional radiation and concurrent chemotherapy in the treatment of stage T2 low-lying adenocarcinomas of the rectum.

A total of 110 eligible patients completed the study protocol (59 with T1 tumors and 51 with T2 tumors). The 6-year overall disease-free survival rates were 85% and 78%. Overall, 9 patients (2 with T1 tumors, 7 with T2 tumors) experienced local recurrence, and 4 patients died of the disease. Perioperative morbidity was minimal. The authors concluded that this treatment approach is reasonable in carefully selected patients.

Late Treatment Failure
Patterns of disease recurrence after local excision with and without postoperative adjuvant therapy provide additional insight into outcomes for patients with T1-T2 tumors. As the data from selected large series of patients who did or did not receive postoperative radiotherapy (Table 5) show, postoperative radiotherapy seems to result in a shift toward later local failure compared with local excision alone. In the combined experience of Massachusetts General and Emory University Hospitals, the median time to local recurrence was 13.5 months for patients treated with local excision alone and 55 months for patients treated with...
postoperative radiotherapy. Paty et al.6 also noted a delayed time to local recurrence for patients receiving adjuvant chemotherapy (2.1 vs. 1.1 years), but added that overall local and recurrence rates were similar for both groups. These study results emphasize the clinical importance of extended follow-up in these patients, and also reinforces the need for studies to evaluate long-term data.

### Salvage Therapy

A number of studies have evaluated treatment options after local recurrence. In their retrospective study of local excision as definitive treatment of predominantly T1-T2 tumors, Graham et al. found local recurrence rates of 19%, but noted that more than half of these patients experienced cure after additional surgery. In examining outcomes among any groups of rectal cancer patients treated with local excision, regardless of the use of adjuvant therapy, a critical question must be addressed: 1. How often can locoregional recurrence be treated effectively with further surgery?

Although the small numbers of patients discussed in published experiences addressing this question can make definitive conclusions difficult, results of attempted salvage for patients with local recurrence after local excision are summarized in Table 6. In these combined series, comprising 493 patients treated, 73 patients experienced local failure either alone or in combination with distant disease. In 44 (60%) of these patients, a potentially curative, margin-negative salvage procedure was performed, most often using APR. Of these 44 patients, 21 (48%) showed no evidence of disease at varying lengths of follow-up.

Salvage therefore seems to be possible in more than half of patients with isolated local failure after local excision. It is important to remember, however, that more than 50% of patients who experience recurrence will eventually die of their disease.

Baron et al. examined the timing of salvage after local excision at the Memorial Sloan-Kettering Cancer Center. They compared the outcomes for 21 patients who had undergone local excision followed by immediate APR or low anterior resection (LAR) for tumors with adverse histologic features with the outcomes for 21 patients who had undergone local excision followed by LAR or APR at the time of clinical local recurrence. Disease-free survival was significantly improved in the patients undergoing immediate LAR or APR (94.1% vs. 55.5%; P < .05).

The results from these studies suggest that the argument for a liberal approach to selecting patients for local excision based on good salvage potential in patients whose disease recurs is not supported by the literature.

### Alternative Therapies

Alternative forms of local therapy for T1 and T2 rectal cancer have been reported, including endocavitary irradiation, fulguration, cryosurgery, and Nd:YAG laser therapy. Of these modalities, endocavity irradiation has received the most attention. In Papillon’s initial experience with this technique in 1972, the local recurrence rate was 7% and the 5-year overall survival rate was 72% among a selected group of patients at low risk for developing recurrence or metastases. The potential advantage of endocavitary irradiation over external beam radiotherapy is the ability to deliver a higher dose of radiation in a more concentrated fashion to the tumor.

Both Papillon and others have subsequently reported similar results, again among highly selected low-risk patients. Birnbaum et al. identified “ideal” characteristics of rectal lesions for treatment by combination endocavitary and external beam irradiation (Table 7). Among 72 patients, they found that recurrence was significantly less likely in patients with “ideal” tumors than in those with non-ideal tumors.

---

**Table 5 Patterns of Local Recurrence After Local Excision With and Without the Use of Postoperative Radiotherapy.**

<table>
<thead>
<tr>
<th>Series</th>
<th>N</th>
<th># LR</th>
<th># LR &gt; 2 years postop</th>
</tr>
</thead>
<tbody>
<tr>
<td>Local Excision Alone</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Chakravarti et al.53</td>
<td>52</td>
<td>10</td>
<td>2 (20%)</td>
</tr>
<tr>
<td>Bailey et al.52</td>
<td>28</td>
<td>2</td>
<td>1 (50%)</td>
</tr>
<tr>
<td>Willett et al.56</td>
<td>40</td>
<td>6</td>
<td>1 (17%)</td>
</tr>
<tr>
<td>Biggers et al.57</td>
<td>141</td>
<td>36</td>
<td>4 (11%)</td>
</tr>
<tr>
<td>Local Excision + Postoperative Radiotherapy</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Chakravarti et al.53</td>
<td>47</td>
<td>8</td>
<td>6 (75%)</td>
</tr>
<tr>
<td>Bailey et al.52</td>
<td>34</td>
<td>2</td>
<td>1 (50%)</td>
</tr>
<tr>
<td>Willett et al.56</td>
<td>26</td>
<td>4</td>
<td>2 (50%)</td>
</tr>
</tbody>
</table>

Abbreviation: LR, local recurrence.
These authors stressed the importance of careful clinical and endorectal ultrasound staging to identify patients most likely to benefit from this treatment approach. All of these studies possible for more than half of patients who experience recurrence after local excision, studies suggest that the potential for cure in these patients is less than was previously expected. In addition, immediate radical resection in patients with tumors found to have adverse histologic features at local excision has shown improved results over radical resection performed after recurrence.

**References**


2. Lipshultz SE, Colan SD, Gelber RD, Perez-Atayde AR, Sallan SE, Sanders SP. Late cardiac effects of doxorubicin therapy...


34. Mukai M, Sadahiro S, Yasuda S, et al. Preoperative evaluation by whole-body 18F-fluorodeoxyglucose positron
Local Excision for Rectal Cancer